The development and validation of a patient-centred quality-of life questionnaire for adult neuromuscular disease

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THE DEVELOPMENT AND VALIDATION OF A PATIENT-CENTRED QUALITY OF LIFE QUESTIONNAIRE FOR NEUROMUSCULAR DISEASE

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ABSTRACT

Quality of life (QoL) questionnaires are important measures of change in clinical trials and as clinical tools they can be used to identify individual problems that may be amenable to intervention. However, to date no disease-specific measures of QoL for neuromuscular disease (NMD) have been developed. Generic measures have been used but they do not look at issues that are specifically relevant in NMD. Therefore, they may miss issues of importance to patients and they may also lack sensitivity to change.

In order to develop a disease specific measure of QoL for NMD, qualitative interviews and a postal survey were conducted to explore patients' experiences. A literature review exploring the QoL concept, the evolution of QoL assessment and difficulties incurred in measuring QoL resulted in the development of a theoretical model of QoL. This was used alongside interviews and survey findings to design a disease-specific QoL questionnaire, the Individualised Neuromuscular Quality of Life questionnaire (INQoL).

The INQoL looks at the impact of key NMD symptoms and the effects of NMD upon specific areas of life. Resulting scores are based upon patients' satisfaction with and the importance they attach to these life areas.

A pilot study demonstrated the face and content validity of the INQoL and its acceptability to patients. Clinimetric evaluation established construct validity and test-retest reliability. A preliminary measure of responsiveness over a 3-6 month period was also obtained. Finally, clinical utility was assessed in a pilot study. This demonstrated its acceptability to both patients and physicians in a clinical setting and potential usefulness as a clinical tool.

Further research will confirm the responsiveness of the INQoL. There are also plans to validate the INQoL cross-culturally and to extend validation to other neuromuscular conditions.

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CHAPTER I

CONCEPTUAL AND MEASUREMENT ISSUES IN QUALITY OF LIFE

Chapter 1: Conceptual and measurement issues in quality of life.

1.1 Introduction.

Quality of life (QoL) has played an important role in health care since the early foundations of medical treatment and ethics. Indeed, patients' point of view about QoL and its relation to health can be traced back over 300 years (Apolone and Mosconi, 1998). However, it is only over the last century that QoL issues have gained more prominence, initially in the fields of politics and sociology but more recently in health care.

In medicine, QoL issues began to receive more attention after the World Health Organisation (WHO, 1948) defined health as "not only the absence of disease and infirmity but also the presence of physical, mental and social well-being". This new definition challenged limitations inherent in the previous, largely negative conceptualisation of health as simply "the absence of disease". The positive connotations of QoL assessment led to its increasing popularity (Gill and Feinstein, 1994; McHorney et al, 1993) and from the mid-1970s QoL assessments started to appear in clinical trials, most notably in the fields of oncology, rheumatology and psychiatry (Farquar, 1995). Since this time, QoL assessment has become increasingly fashionable. However, there has been considerable uncertainty over the pertinence and usefulness of the data generated using existing QoL measures.

In order to determine the relevance of QoL research and its role in health care it is important to explain the rationale behind QoL assessment, to define 'quality-of-life' and to determine whether or not it is possible to measure QoL.

1.2 Why Measure QoL?

Over the last century, dramatic improvements in health care have led to increases in life expectancy and a consequent rise in the number of people living with chronic conditions. As a result it has become inappropriate to use traditional measures of outcome. Mortality statistics are relevant in acute illness in which the main aim of treatment is to prolong or save life. However, for those living with a chronic illness or disability, treatment is directed more towards improving QoL (Bowling, 1997). Biological (e.g. muscle biopsy, CK level) and functional tests (e.g. muscle strength and performance in neuromuscular disease) may not reflect important factors that can be affected by medical or social interventions (e.g. QoL or disability). It has therefore become necessary to conduct assessments of subjective factors in order to understand the effects of disease within the context of patients' lives. Issues such as life satisfaction, relationships with family members and the ability to socialise (Drummond, 2000) have been incorporated into assessments. These are more relevant to patients and may be particularly useful in determining the efficacy of certain interventions.

1.3 The Paradox of Quality of Life.

The difficulty with using QoL measures to evaluate treatment and overall health is that QoL scores often contradict clinician's ratings of health status (Slevin et al, 1988; Rothwell et al, 1997) as well as objective measures of health or physical function (Addington-Hall and Kalra, 2001). For example, unexpectedly high levels of QoL

have been reported in studies of chronically ill and disabled patients (Burckhardt et al, 1989; Lundqvist, 1991; Strombeck et al, 2000). Similarly, cancer patients have reported levels of satisfaction comparable to healthy comparison groups (Watson and Pennebaker, 1989; Fromm et al, 1996; Taylor et al, 1984).

In a study of patients with moderate to severe disabilities, 50% reported having either an excellent or good quality of life (Albrecht and Devlieger, 1999). These unexpectedly high levels of QoL were reported as a 'disability paradox'.

These findings of disparity between QoL findings and the clinical picture have meant that the usefulness of QoL assessment is uncertain, as it is difficult to determine the value of treatments if the outcome changes little despite changes in the clinical picture.

These findings have led researchers to examine the concept of QoL in greater depth and have promoted the development of QoL theories with advances in QoL measurement.

1.4 What is Quality of Life?

The main problem in assessing QoL is the widely acknowledged lack of consensus on its definition (Bowling and Brazier, 1995). This has made it difficult to measure QoL in a comprehensive and efficient manner and to generalise between QoL studies (Gill and Feinstein, 1994). The tendency to class any non-clinical measurement under the QoL umbrella (Bowling, 1997) and include QoL measures as an afterthought in

clinical trials (Bergner, 1989) has further exacerbated this problem.

The abstract and subjective nature of QoL lies behind the problems in its conceptualisation (Gill and Feinstein, 1994). However, a number of definitions of QoL and health-related quality of life (HRQL) converge on the idea that QoL depends upon the perceived distance between an individual's current state and the ideal state to which s/he aspires (table 1.1). The influence of the social and physical world on QoL has also been acknowledged (Elkinton, 1966; Orley and Kukyen, 1994; WHOQoL Group 1995).

The introduction of the health related quality of life (HRQL) construct demonstrated a move to enhance the specificity and usefulness of QoL assessment in health care. However, like the QoL concept, HRQL is seldom defined in the literature (Bowling, 1995) and it is arguably no less obscure than QoL considering that almost all areas of life are in some way 'health-related'. For example, mobility restrictions not only influence functional capacities, but also social and family life, occupational life and financial status. The definitions of HRQL presented in table 1.2 also imply the importance of a state of harmony or equilibrium.

The idea of QoL as the gap between an individual's current state and how they would like to be presents QoL as a conceivably measurable entity. These definitions acknowledge the dynamic nature of QoL and take into account the influence of the situation (e.g. ill health) and each individual person and his/her goals.

Table 1.1: Definitions of quality of life

• "Harmony within a man, and between a man and his world" (Elkinton, 1966)
• "The degree of satisfaction of human needs" (Hornquist, 1982)
• "Individuals' perceptions of their position in life in the context of the culture and value systems where they live and in relation to their goals, expectations, standards and concerns" (Orley and Kukyen, 1994; WHOQoL Group, 1995)
• "The extent to which our hopes and ambitions are matched by experience" (Calman, 1984)
• "Appraisal of one's current state against some ideal" (Cella and Tulsky 1990; Cella and Tulsky 1993)
• "The discrepancy between ideal and real states" (Campbell et al, 1976)

Table 1.2: Definitions of health-related quality of life

•"The best possible physical and emotional state compatible with [a patient's] medical condition" (Leplege and Hunt, 1997).
• "A patient-centred focus relating to the impact of a perceived health state on the ability to lead a fulfilled life" (Price, 1996)

1.5 Goal Fulfilment and Emotional Equilibrium: Discrepancy Theories

The relationship of QoL to a perceived discrepancy between current state and an individual's internal standard of comparison is compatible with the *multiple discrepancy theory (MDT)* of satisfaction (Michalos, 1985) and the *self-discrepancy theory* of emotion (Higgins, 1987).

1. The *MDT* states that people compare themselves to multiple standards of reference. These include the individual's own past conditions, their aspirations and ideal levels of satisfaction, the needs they wish to fulfil and goals they wish to achieve, as well as the situations of other people. Comparison with these standards

results in a degree of satisfaction based on the discrepancy between the individual's current condition and these standards.

2. In *self-discrepancy theory*, emotional reaction is believed to depend upon the discrepancy between a person's current state and either their ideal state or their 'ought' state (how others believe a person should or ought to be). Upwards comparison (with a standard higher than the individual's own state) is thought to result in a decrease in satisfaction and downwards comparison (with a standard lower than their current state), in increased satisfaction.

The dependence of QoL upon this type of discrepancy has been documented in recent research on QoL in disability. Albrecht and Devlieger (Albrecht and Devlieger, 1999) propose that self-image is impaired when there are discrepancies between what we would like to do and what we can do, or between what we used to be able to do and what we now can do. By closing such gaps, through a reduction in the constraints of the environment or by enhancing an individual's physical capacity, it is believed that a 'balance between mind, body and spirit' can be achieved, thereby improving QoL.

1.6 Minimising discrepancy and achieving high QoL

Perceived 'control' is important to QoL (Taylor, 1983; Heyink, 1993) with patients who take control and introduce order into their lives reporting high levels of QoL. These patients can actively move towards and fulfil aspirations by learning what is possible and setting realistic goals. Conversely, patients who report difficulties in managing their impairments, have been found to report lower levels of QoL (Albrecht and Devlieger, 1999)

Narrowing perceived discrepancies can occur through an actual (bottom-up) change or

a perceived (top-down) change in the situation (due to psychological processes) (Diener, 1984). 'Bottom-up' changes that might result in an improvement in QoL include:

- An improvement in the patient's physical capacity
- A reduction in the constraints of the environment
- An increase in the patient's social support

'Top-down' changes that might result in an improvement in QoL include:

- A lowering of expectations
- The setting of more realistic goals

1.6.1 The Bottom-Up Approach

The 'bottom-up' approach to QoL is based on the idea that there are basic and universal human needs (e.g. need for food, warmth and social contact) which, if fulfilled, result in positive well being. This parallels the more traditional medical view that QoL depends upon an individual's physical state and that those with more severe illness or disability will have poorer QoL due to greater difficulties in fulfilling the roles and functions expected of 'healthy' individuals. Advocates of this approach believe that addressing these 'universal' human needs through the use of particular medical interventions will improve QoL.

Maslow's hierarchy of needs (Maslow, 1962; Maslow, 1970) can be equated with this approach. This states that the needs at the bottom of the hierarchy must be met,

namely the need for warmth, food and safety before an individual can pursue satisfaction in other areas of life. The higher needs delineated by Maslow, such as the need for acceptance, love, esteem and ultimately self-actualisation (being completely true to one's own nature) are likely also shared. However, the way in which these needs are fulfilled is likely to differ between individuals. Therefore, there is no agreed formula for the fulfilment of individual needs. This presents difficulties in accurately capturing QoL and formulating effective interventions to improve QoL.

1.6.2 The Top-Down Approach

The 'top-down' approach to QoL, or the idea that psychological processes act to alter internal standards and accommodate changes (e.g. the onset of disease) can be used to explain many unexpected findings in QoL studies.

These psychological processes are widely studied in relation to happiness and well-being (Headey et al, 1984; Wilson, 1967), which like QoL appear to be remarkably stable despite changing circumstances. Changes in QoL are widely believed to depend upon the influence of coping and adaptation (Lundqvist, 1991; Muldoon et al, 1998). These influences are particularly important in the QoL evaluations of patients with chronic conditions (Cassileth et al, 1984; Breetvelt and van Dam, 1991).

1.6.2.1 Coping

Coping strategies are used in order to achieve a balance, or a match, between the patient's perceived state and an internal standard of reference (e.g. the individual's ideal or the norm of the surrounding social world) (Taylor and Lobel, 1989; VanderZee and Buunk, 1995; Gibbons, 1999). Patients adopt diverse coping strategies

that depend upon factors such as personality, the situation and the resources available (Lazarus and Folkman, 1984; Folkman, 1997). These strategies include seeking social support (Taylor et al, 1986; Sarason et al, 1985) and engaging in spiritual practice (Park et al, 1990).

Problem-focused coping tends to involve action on the external world (e.g. fitting a stairlift to make moving between floors at home easier), whereas psychological coping strategies include reframing expectations (Heyink, 1993; Allison, 1997), and reordering goals (Rapkin, 1994). For example, goals might be reorganised in order to maintain the self-concept by downgrading the importance of domains that are a constant source of negative emotion (Taylor and Brown, 1988; McCrae and Costa, 1988).

1.6.2.2 Adaptation

Adaptation, described as "psychological recuperation after a setback" (Heyink, 1993) is important in helping individuals to regain and maintain acceptable levels of well-being. It is believed to take place through the process of 'habituation' (Helson, 1964), in which the reactions of the emotion system to new events gradually dampens over time.

Many findings show that adaptation plays an important role in the well-being of people with disability or ill health. For example, time since injury positively predicts general satisfaction (Krause and Sternberg, 1997) and patients with congenital disabilities report better adjustment than those who acquire disability later on in life (Li and Moore, 1998). Adaptation complicates QoL measurement, as it means that QoL is likely to remain relatively stable over time despite apparent changes in

physical health.

Of course, the individual situation or the stimulus may influence the rate of adaptation. For example, adaptation to the effects of a sweeping event, such as the onset of a very disabling condition is likely to take a long time because of the long-term effects that this is likely to have on daily life (Suh et al, 1996). Disabling conditions may impinge upon a wide variety of goals making adjustment to other goals more arduous (Deiner et al, 1999). Similarly, individual differences between people and differences in their circumstances influence adaptation and the coping strategies adopted. This makes it difficult to interpret QoL findings without considering the life context of the individual.

1.6.2.3. The Influence of Personality

Another explanation for the disparity between patients' reported experience of health and the clinical picture is the influence of personality upon the evaluation of events. Knowledge of problems is likely to change more over time than evaluations of health status. This is because emotional evaluations are influenced by personality which means they are therefore likely to remain relatively stable (Lazarus and Smith, 1988). In fact, personality has been found to be one of the strongest and most consistent predictors of subjective well-being (Gill and Feinstein, 1994; Bullinger, 1999; Costa and McCrae, 1984). Well-being may even depend more on personality than upon the individual's positive and negative experiences (Costa and McCrae, 1980; Brickman et al, 1978; Diener 1984).

However, this does not explain findings of significantly lower levels of happiness in patients with spinal cord injury compared to those in a 'healthy' control group

(Brickman et al, 1978). Nor does it explain findings of more negative psychological reactions in more severely disabled patients (Viney and Westbrook, 1981). This fluctuation of well-being over time and the influence of external events upon satisfaction suggests that recent life events interact with personality to influence overall well-being (Brief et al, 1993; Feist et al, 1995).

1.6.2.4 Goal restructuring, response shift and its influence on well being

An inability or expected inability to satisfy certain goals may lead goal restructuring. For example, the importance attached to goals and the content of these goals may change (Rapkin, 1994), new goals may emerge (Hyland, 1998), and some goals may be abandoned altogether. This process may lead to improved levels of well-being as more achievable goals are adopted.

Goal restructuring has been described in the extensive literature on response shift as the process believed to underlie unexpected changes in QoL evaluations. Three types of response shift have been proposed (Sprangers and Schwartz, 1999). These influence QoL through :

1. recalibration of the internal standards to which the patient compares his/her current state
2. a change in the conceptualisation of goals (or content of goals)
3. a change in the importance/value attached to goals

This framework aims to elucidate the factors that underlie changes in QoL. It is proposed that demarcating aspects of response shift in QoL measures will indicate whether changes in reported QoL relate to changes in the patient's state of health,

their functional ability or to changes in the patient's conceptualisation of QoL.

1.7 Does Goal Fulfilment Lead to Positive Well-being?

Defining QoL as the distance between the individual's current and ideal state of being presents a neat and measurable entity. However, it is apparent that the closure of this 'gap' may not result in well-being and that further psychological processes may be at work.

1.7.1 Value of the goal

As delineated in the response shift literature (Sprangers and Schwartz, 1999), individual patients attach differing amounts of importance to the relief of various complaints and to particular goals or life areas (Wright et al, 1994). This means that goals or areas of life that are more important to individual patients are likely to have a greater bearing on QoL. Consequently, a discrepancy between an individual's current and ideal state in one of these areas is more likely to have a negative effect than a similar discrepancy in an area that is of little consequence to the individual. For example, a 65 year old woman who has always been very family oriented may be more upset about not being able to pick up her grandchildren than by limitations to her leisure activities.

1.7.2 Expectations

Regardless of the gap between a patient's current state and the state to which s/he aspires, expectations about whether they will achieve their desired state depend upon factors such as life situation (Scheier and Carver, 1987) and past success in fulfilling goals (Csikszentmihalyi, 1990). Expectations brought about by medical prognosis also influence QoL. For example, a study of patients who had been severely injured in an

accident but who had a good prognosis reported high levels of reported QoL (Schnyder et al, 1999). It may be that as these patients were optimistic about future recovery there was no need for them to adapt or adopt any long-term coping strategy (Schnyder et al, 1999).

The more negative expectations that are likely to be held by patients with chronic progressive disorders may also play a significant role in QoL, particularly if the patient is apprehensive about the prospects of future deterioration. Conversely, uncertainty is also believed to play an important role in coping (Allison, 1997), and in the maintenance of hope (Padilla et al, 1992).

Realistic expectations may play an important part in maintaining well-being (e.g. the knowledge that a particular treatment will relieve pain in the long term). However, changes in expectations complicate the interpretation of QoL scores. For example, patient expectations may improve following a successful intervention meaning that subsequent measurements of QoL are unlikely to reflect similar beneficial effects due to parallel improvements in both current and expected condition (Macduff, 2000).

1.7.3 Progress and rate of progress

According to the theory of hedonic relativism it is human nature to strive towards ever higher aspirations (Brickman and Campbell, 1971). In fact, the process of moving towards or away from one's goals may be more important than the actual achievement of these goals (Hsee et al, 1991; Hsee and Abelson, 1991). Furthermore, it has been proposed that when efforts to achieve a particular goal are disrupted, individuals stop to assess the likelihood they will achieve their goal, taking into account physical or

social constraints as well as the resources available to them. This implies that the reference against which behaviour is compared may not be the final desired outcome but the actual process leading to the achievement of a goal.

The rate of progression towards or away from aspirations may also be important to patients' subjective well-being (Hsee et al, 1991). Therefore, the acceptable or desired rate of discrepancy reduction may be another standard of reference with which the individual compares their current rate of progress (Carver et al, 1996).

1.8 Frameworks of Disability and QoL

1.8.1 QoL within the context of the ICIDH-2

It has been suggested that the World Health Organisation's "International Classification of Impairments, Disabilities, and Handicaps' (ICIDH; WHO, 1980) provides a more complete description of disease consequences and health outcomes than do generic HRQL measures (Ebrahim, 1995). This may be because it demarcates the different levels of disease in a framework that can be used to explain the underlying determinants of QoL, and upon which goals for rehabilitation can be based.

The multidimensional relationship between the three levels of disease impact (I, D, and H), previously represented in an interactive model (Carr and Thompson, 1994) is recognised in the revised ICIDH model (ICIDH-2) (Bickenbach et al, 1999) (Gray and Hendershot, 2000). This revised model renames the levels of 'Impairment, Disability and Handicap' as 'Impairment, Activities and Participation' respectively. These

represent the direct result of disease or disability upon structure or function at body level (Impairment), the restriction or lack of ability to perform activities at the level of the individual (Activities) and the disadvantage and role limitation to the individual in a social context (Participation).

The model incorporates the facilitators and barriers that encourage or impede an individual's involvement in the activities typical of "healthy" individuals in their society. It thereby captures the impact of the condition in the context of patients' lives and the environment in which they live. This makes it a good starting point for practical intervention and therapeutic action beyond purely medical interventions.

1.8.2 The Quality of Life "Process"

Many QoL questionnaires contain items about symptoms and functioning within various life domains as well as items that relate to satisfaction and general well-being, all of which are important to the overall picture of QoL. The aggregation of scores into an overall QoL index or separate domain scores does not always allow investigators to differentiate between the determinants of QoL (e.g. symptoms, disease- and symptom-related impact) and QoL itself.

QoL has more recently been approached as a process rather than as an outcome (Leventhal and Coleman, 1997; Hyland, 1992;Wilson and Cleary, 1995). These approaches seek to separate out and thereby better understand the determinants of QoL. The process model (Leventhal and Coleman, 1997) includes:

1. a. Symptoms, emotions and functioning as experienced by patients

   b. Patients attribution of these symptoms, emotions and functioning to disease or treatment

2. Patients' interpretation of their physical and emotional sensations

3. The integration of these assessments (e.g. experienced symptoms, emotions and the interpretations of these physical and emotional sensations) into overall QoL judgements.

Hyland presents a similar model that represents QoL as a causal sequence of symptoms and anticipated symptoms, the problems caused by these and patients' evaluation of these symptoms and problems (Hyland, 1992). Psychological factors are also introduced into the model. These include the interpretation of symptoms, coping style and final evaluations of these problems and symptoms.

By separating out these underlying factors these models aim to clarify how health professionals can use HRQL data to guide and improve therapeutic interventions (Wilson and Cleary 1995; Carr and Higginson, 2001). Questionnaires based on these models should highlight problems that may be amenable to treatment and indicate the most appropriate intervention (e.g. drug treatment, advice, occupational therapy, support from social services). They could also provide an idea of how the patient is coping with his/her condition and help in the assessment of interventions by separating out the effects of adaptation and coping from the direct effects of treatment.

1.9 A Proposed Model of Health-Related Quality of Life

From the findings about the influence of illness and disability upon QoL with reference to psychological, social and physical factors, a theoretical model of QoL

was developed (Figure 1.1). This is based upon 4 main frameworks.

1. The discrepancy framework

2. Response shift

3. The ICIDH-2 (a disability rehabilitation framework)

4. Process accounts of QoL

The ICIDH-2 section of the model comprises the Impairment, Activities and Participation boxes, each of which may be influenced by an intervention. How individuals are affected in these three areas determines their overall current state, which also influences their expectations.

Expectations are also influenced by the individual's internal standards (e.g. how they have been in the past or their perceptions of other people) which in turn influences the individual's goals. The perceived discrepancy between the individual's current state and their goal or ideal state and their progress towards this determines the individual's QoL. This evaluation brings about response shift. Through this the individual's internal standards or the content of their goals may change with reductions in the discrepancy between current and goal state leading to improvements in QoL. They may also attach a different value to these goals and thereby change the degree of influence of a perceived discrepancy upon QoL.

This model provides a good theoretical basis on which to develop a QoL questionnaire as it encapsulates QoL-as-process models by incorporating disease impact at the levels of the ICIDH-2, as well as the psychological influences that act to regulate overall evaluations of QoL. Such a framework should facilitate the interpretation of scores

Figure 1.1a. Proposed model of health-related quality of life

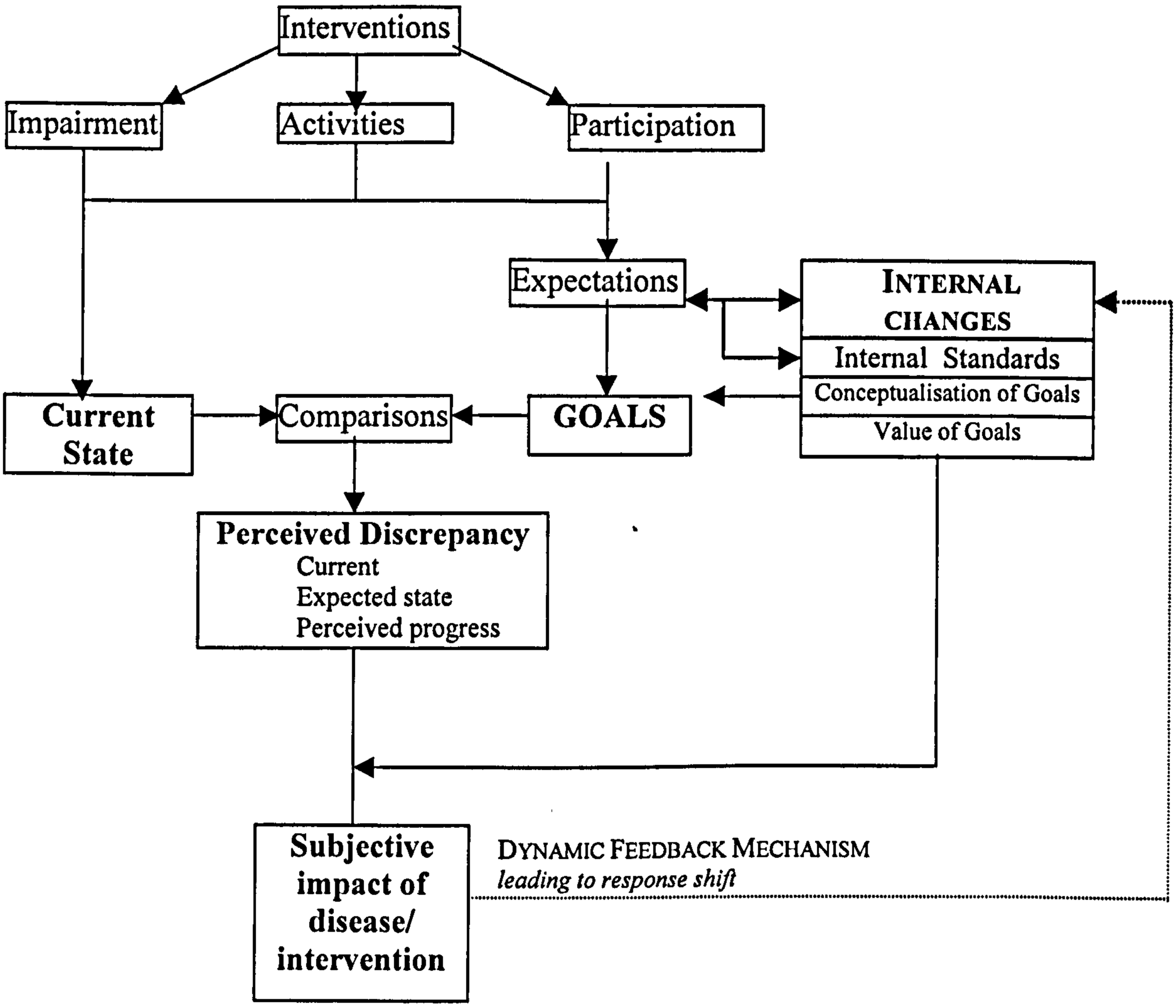
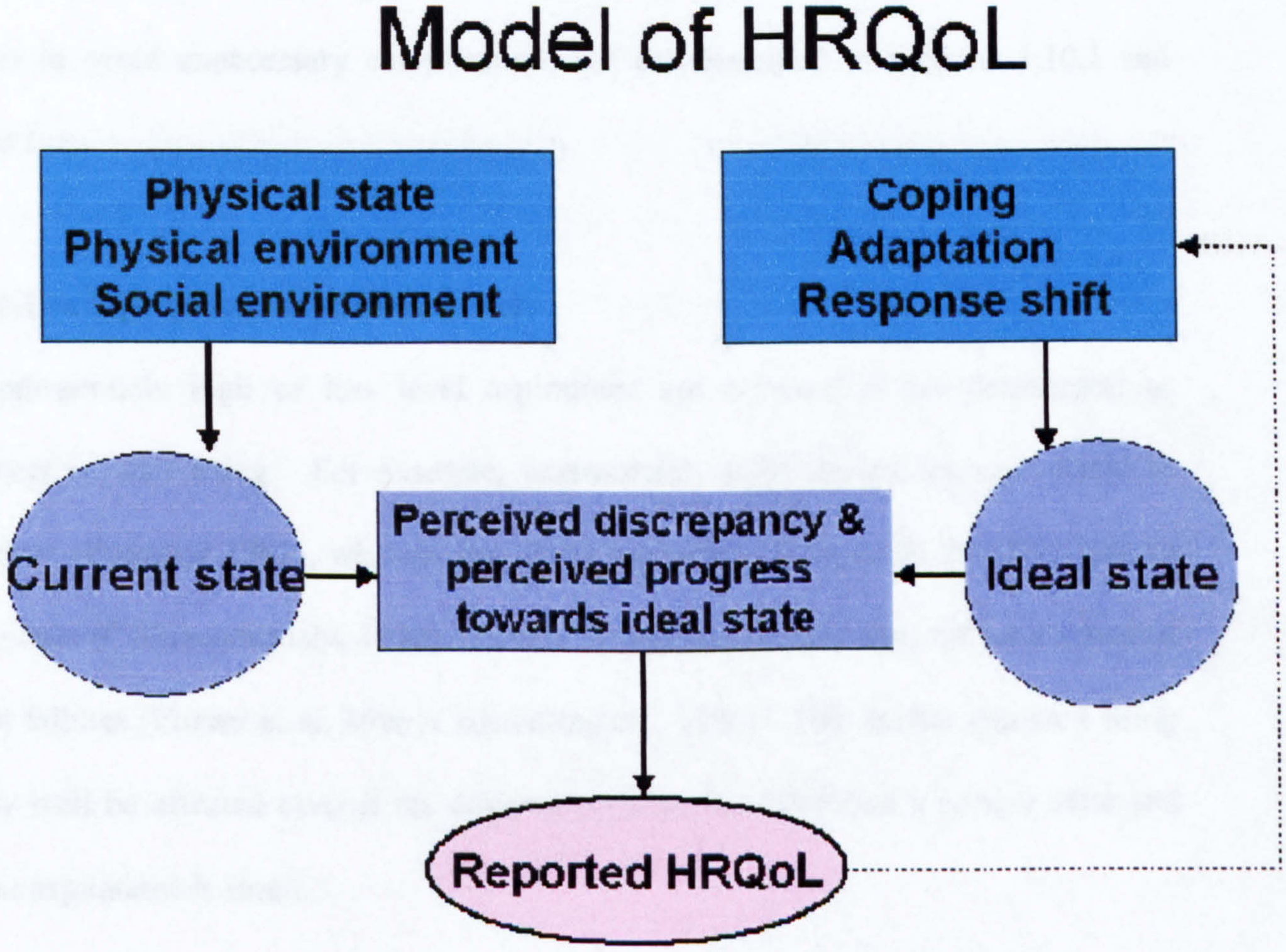


Figure 1.1b. Simplified model of health-related quality of life



derived from the scale making it easier to determine the underlying determinants of an individual's QoL.

1.10 Additional QoL Determinants not Employed in Proposed Theoretical Model

Numerous other psychological processes are likely to influence the perception or experience of QoL. These processes were not incorporated into the framework in order to avoid unnecessary complication, but are described in sections 1.10.1 and 1.10.2.

1.10.1 Inappropriate Aspiration Level

Inappropriately high or low level aspirations are believed to be detrimental to subjective well being. For example, unreasonably high aspirations are related to anxiety (Emmons 1992), whereas low-level aspirations have been found to lead to boredom (Csikszentmihalyi 1990). Low level aspirations may also reflect a series of past failures (Carver et al, 1996; Csikzentmihnly, 1990). This means that well-being may well be affected even if the distance between an individual's current state and their aspirations is small.

Similarly, goal restructuring may not always result in increased QoL levels. In chronically ill patients, the abandonment of valued goals may eliminate the discrepancy between real and ideal states. However, if this also results in a reduction in the overall level of positive emotion and motivation there is unlikely to be an improvement in QoL (Rapkin, 1994).

The theoretical model outlined by Hyland (Hyland, 1992) proposed two separate affective systems, positive well-being and negative well-being. He also proposes that

in order to maintain emotional equilibrium individuals avoid situations in which failure is likely or the possibility of fulfilment is low. Therefore, although they may minimise negative well-being by abandoning goals and avoiding failure, their chance of experiencing positive events and enhancing positive well-being would simultaneously be reduced.

1.10.2 Type of Aspiration

The nature of the individual's circumstances and their aspirations are also important to QoL. For example, the fulfilment of realistic goals that relate to intrinsic needs are likely to have a positive impact upon QoL. However, the fulfilment of extrinsically motivated desires may ultimately be less satisfying (Emmons 1992; Kasser and Ryan 1996; Maslow 1970; Diener et al, 1999; Csikszentmihalyi, 1990). For example, an individual who has a job in which s/he feels valuable and is respected by his/her colleagues is likely to have a higher level of QoL than someone who earns more (extrinsic motivation for financial wealth) but does not feel valued or respected for what they do.

These findings correspond to the idea that in order to improve QoL basic and universal needs must first be fulfilled before striving towards other goals. Nonetheless, the specific kinds of aspirations held within a 'hierarchy of needs' (Maslow, 1970) are likely to differ depending upon the personality and situation of the individual.

1.11 Conclusion

QoL assessment represents an important move away from more objective measures of health such as morbidity and mortality. These measures provide insufficient

information about the impact of disease in the context of patients' lives, an issue that has become increasingly important as people are living longer and facing chronic illnesses that may impede their ability to lead fulfilled lives. The subjective experience of illness is assessed through the administration of QoL measures.

The importance of assessing QoL is widely acknowledged. However, it has proved to be a difficult task given the difficulties involved in conceptualising the construct such that it provides useful information within the context of health care.

The most useful and intuitive definitions of QoL are based around the idea that QoL depends upon patients' perception of the degree to which his/her aspirations are fulfilled and the perceived possibility of fulfilling them. In the context of disease impact, this translates into a conceptualisation of HRQL as the degree to which the patient perceives the fulfilment of her aspirations to be influenced by her health.

However, QoL is not only influenced by the patients' ability to fulfil their aspirations or goals. Other factors that play an important role include the individual's progress and rate of progress towards their aspirations, their expectations (e.g. based on past success in achieving goals or medical prognosis) and their ability to recalibrate the scale used to judge their current state. The importance attached to goals also influences their contribution to perceived QoL.

QoL study findings and theories were discussed and have been incorporated into a model of HRQL. This framework incorporates the major aspects of discrepancy theories of QoL, theories of response shift, the ICIDH-2 and process accounts of QoL. It aims to present a comprehensive model that delineates the way QoL is evaluated by the individual, incorporating both internal and external influences and how they

interact (e.g. expectations and changing internal standards). The interaction between personal characteristics and the individual's social and physical environment influence evaluations of QoL. By understanding this interaction we can improve QoL assessment. The possibility of using the ICIDH-2 or "biopsychosocial" model alongside QoL questionnaires may help to guide intervention and rehabilitation.

CHAPTER II

QUALITY OF LIFE QUESTIONNAIRES

Chapter 2: Quality of life questionnaires

2.1 Introduction

Due to the lack of confidence in the accuracy and reliability of responses to the informal question of 'how are you feeling?' (Parkerson et al., 1992), standardised QoL questionnaires have increasingly been adopted (Ware, 1993; Jones, 1997). However, many QoL studies are impeded by poor design and insufficient assessment methods (Bowling, 1997). For example, in a review of the face validity of 75 articles purporting to report QoL investigations, it was found that more than half the articles examined did not mention the term 'quality of life' (Gill and Feinstein, 1994). Further findings showed that 15% of the studies reviewed did not provide the investigators' conceptualisation of QoL, 47% did not identify the specific domains incorporated in the QoL assessment and 36% did not explain their choice of QoL instrument.

This lack of consistency in QoL assessment and reporting and differences in the rationale, development and content of existing QoL measures make it difficult to compare and generalise across different studies.

Despite this, there is increasing evidence about the reliability and validity of QoL measures (McDowell and Newell, 1996) as well as numerous clinical trials that demonstrate the ability of these measures to demonstrate clinically important change (Croog et al., 1986; Bombardier et al., 1986; Tugwell et al., 2000).

In order to get an idea of the differences between measures and of their merits and drawbacks a number of the key QoL questionnaires currently in use will be discussed. This will lead on to a more specific discussion of QoL assessment in NMD to date.

2.2 Types of Quality of life questionnaire

The two main types of QoL scale are generic and disease-specific measures. These have different properties which make them suitable for use in different kinds of investigation (Price, 1996).

2.2.1 Generic measures

Generic instruments are developed to assess a wide range of domains and can be applied to a diversity of health states, diseases, and conditions. They are usually developed over many years, and can therefore be used with more confidence in their validity than newly developed specific questionnaires.

Generic questionnaires can be used to derive norms for age and sex when used in population surveys (Price, 1996). This makes them useful in making comparisons between different disease groups and conducting health surveys in population or patient groups (Fletcher et al., 1992). They also include domains that may not typically be included in disease-specific measures and can therefore detect unexpected effects. However, as they tend to be less concerned with clinical matters than disease-specific measures they may be less suitable for use in clinical practice (Troidl, 1991). As they do not pick up more specific areas of concern they often also lack sensitivity to clinically relevant change (Fletcher et al, 1992; Price, 1996).

2.2.2 Disease-specific measures

In order to address the shortcomings of generic QoL questionnaires, a large array of disease-specific instruments started to emerge during the 1980s. These ranged from

those developed for hypertension and heart disease (e.g. Croog et al, 1986), to those for arthritis (Bombardier et al., 1986).

These measures include only relevant dimensions which increases their intuitive relevance making them more acceptable and less of a burden to patients (Fletcher et al., 1992). They also have greater responsiveness than generic questionnaires given their particular relevance to the patients under study (Bessette et al., 1998; Bradley et al., 1999) (Sprangers and Aronson, 1991).

The acceptability of these measures and their sensitivity to change means that they are useful for monitoring patients progress over time in clinical trials and in clinical practice (Bech, 1993). However, they cannot be used to compare groups with different conditions, which decreases their usefulness in evaluating interventions that are not disease specific (e.g. different nursing methods). They may also overlook effects in dimensions that are not included but may be covered in more general questionnaires (Fletcher et al, 1992).

2.2.3 The complementary roles of generic and disease-specific measures

Generic and disease-specific scales play complementary roles in QoL assessment (Task Force of the MGFA, 2000). It is therefore widely recommended that both types of scale be used in QoL studies (Garratt et al., 1996; Fletcher et al, 1992; Bowling and Brazier, 1995). Disease-targeted scales can provide unique information to supplement areas overlooked by generic questionnaires (Vickrey et al., 1997).

Modular instruments have also been promoted (Padilla et al., 1990; Vickrey et al.,

1997). These provide a core base of norm-referenced questions relevant to many different diseases and populations. Condition-specific modules and questions can be added to this core set of questions as required.

2.3 Standardised generic indices of QoL

2.3.1 Utility Measures

Utility measures of QoL are derived from economic and decision theory to reflect patients' treatment preferences. The preferences (trade-offs or gambles) may come directly from patients' valuation of their own health state, or via ratings of hypothetical states of health (Feeny et al., 1996).

The utility measures used to calculate QoL include econometric tools such as the time-trade off, the standard gamble or rating scale methods. The standard gamble involves patients choosing between living for rest of their lives in a particular health state (e.g. current health) and gambling on an imaginary treatment that could result in either a favourable or an unfavourable outcome (e.g. perfect health or death). For the time-trade-off method patients are asked to make tradeoffs between a shorter life span in perfect health state versus a longer life span in a particular health state (e.g. current health). Rating scales require respondents to rate their current health on a scale (e.g. visual analogue scale) that ranges from the worst (0) to the best imaginable health state (100). For each of these methods the respondents score is converted into a number between 0 and 1. These utility measures are then used to derive quality adjusted life years (QALYs) (years of life adjusted for the decrement in quality of life), summary measures that incorporate quantity of life as well as HRQL. These are calculated by weighting the amount of time spent in various health states by the utility

value that reflects the desirability of that state. These are used to justify the use of resources for treatment.

Indirect methods of utility assessment include the Quality of Well-Being (QWB) Scale (Kaplan et al., 1976), the Health Utilities Index (HUI) (Feeny et al., 1996) and the EuroqoL (Euroqol Group, 1990).

The HUI systems (of which there are three) provide a more comprehensive approach to measuring health status. These use questionnaires to gain information about the type and extent of the disabilities and to obtain utility scores providing information about the relative importance of the disabilities (Furlong et al., 2001).

The QWB scale (Kaplan et al., 1976) combines mortality, morbidity and the benefits/side-effects of treatment into a single summary score that represents the outcome of treatment as QALYs. However, its length and necessary administration by a trained interviewer makes it impractical for use as a customary clinical tool (Carr et al., 1996).

The EUROQoL (Euroqol Group, 1990) assesses perceived health in five dimensions; mobility, self-care, pain, usual activities and psychological status with items weighted according to valuations provided by the general population. Respondents are also required to give an overall evaluation of health status on a visual analogue scale assessment (represented by a 'thermometer').

The brevity of the EUROQoL means that responder burden is minimised and it is therefore more likely to be completed in full be respondents. However, it does not

include important dimensions such as energy and social relationships (Jenkinson et al., 1997) and has limited response options, which means that the scale may be insensitive to change and unacceptable to many respondents.

There are a number of limitations associated with the use of utility measures. Firstly, they have been found to show only a modest correlation with health status measures (Tsevat et al, 1994). This may be because they capture factors other than HRQL, such as risk aversion, value attached to life and attitudes towards particular types of medical treatment (Wilson and Cleary, 1995). There is also concern that the hypothetical questions about abstract health states used in utilities assessment may not be a sound basis on which to rest economic decisions about health care as individuals' values tend to change with their situation. For example, patients with severe or critical conditions have been found to rate death as the worst possible health state whereas healthy individuals rate 'unconscious' and 'worst health state' as worse than death (Badia et al., 1995).

2.3.2 QoL Profiles

Other than utility measures that result in a single score (index) of QoL there are a number of questionnaires that that include questions about different areas or domains of life that result in several individual scores representing each area of life.

For example, the Nottingham Health Profile (NHP) (Hunt and McEwan, 1980; Hunt, McEwan et al., 1985; Hunt and McKenna, 1989) has gained widespread use in both medical and non-medical settings, in its original form and in translated versions. The NHP provides a profile of six scores, representing emotional reactions, energy, pain,

physical mobility, sleep and social isolation.

The first half of the questionnaire represents only severe problems giving rise to floor effects, as individuals who are in good health or only mildly affected are inclined to affirm few statements. This results in a highly skewed distribution of scores with a disproportionate number of respondents scoring at the low end of the scale (Brazier et al., 1992), therefore limiting the accuracy and responsiveness of the measure (Hunt et al., 1985).

The use of individual weightings on NHP items has also been cast into doubt as there have been high levels of correlation between unweighted and weighted scores (Jenkinson, 1991).

The Sickness Impact Profile (SIP) (Bergner et al., 1981) and its UK version the Functional Limitations Profile (FLP) (Patrick and Peach, 1989) measure ill health in relation to its impact on behaviour and daily activities and has been used as a standard with which to appraise other QoL instruments. The SIP includes 136 items that measure 12 dimensions of health, putting it among the longest of the generic measures. However, as the questionnaire takes an average of 30 minutes to complete it is often impractical for use in QoL studies. It has also been argued that there are inconsistencies in its scoring system and a new system has been proposed (Pollard and Johnston, 2001). Restrictions have also been demonstrated in the responsiveness of the SIP. For example, it is not sensitive enough to monitor disease progression in individual patients with motor neurone disease (MND) (McGuire et al., 1996). This has led to the development of a modified disease-specific version for MND (McGuire et al., 1997). Other disease-specific measures have also been derived from the SIP for

stroke (van Straten et al., 1997) and rheumatoid arthritis (Sullivan et al., 1993).

The MOS Short Form 36 (SF-36) (Ware and Sherbourne, 1992) is a 36-item subset of the measures originally developed for the RAND Medical Outcomes Study (Jenkinson et al., 1996). Currently the measure of choice, it has gained widespread use in both clinical and research settings. It measures subjective heath status on eight dimensions; physical function, role limitations, social function, mental health, energy/vitality, pain, health perceptions and change in health. Its brevity, acceptability to patients, coverage of a wide range of life areas affected by illness and its established properties of validity make it an attractive choice of measure (Brazier et al., 1992). However, its brevity but coverage of a wide range of areas means that detailed information may be lost making the instrument susceptible to floor and ceiling effects (Murrell et al., 1999). This loss of precision may be acceptable in large-scale population surveys but not smaller scale clinical research where detailed information is necessary in order to detect clinically important change (Carr et al., 1996).

2.4 Individualised/Patient Centred Questionnaires

The domains of standardised scales often only partly tap the relevant information (Bowling, 1995) and incorporate items that are superfluous to many other respondents. This means they are often less sensitive to change than individualised measures that are designed to pinpoint the areas of most relevance and importance to patients (Feinstein et al., 1986; Bech, 1993).

A number of individualised measures have emerged over recent years, most notably the Schedule for the Evaluation of Quality of Life (SEIQoL; O'Boyle et al., 1992) and

the Patient Generated Index (PGI) (Ruta et al., 1994). These individualised instruments ask the patient to generate areas of life that have the most relevance and importance to their QoL. As these scales are customised to individual patients they tend to be more responsive to change (Jones, 1997). The avoidance of irrelevant questions also saves the additional burden imposed by longer questionnaires.

2.4.1 The SEIQoL

The Schedule for the Evaluation of Quality of Life (SEIQoL) asks respondents to list the five most important areas of their lives (cues) in relation to their overall QoL. They are then requested to rate their status on each cue on a visual analogue scale (VAS). Relative weights of importance are attached to each cue, using one of the two available methods of weighting.

The original interview-based version of the SEIQoL has a complicated weighting system, derived from a decision analysis technique called judgement analysis (O'Boyle et al., 1992; Browne et al., 1994; Murrell et al., 1999). The relative weight of each nominated area is determined by presenting respondents with the profiles of 30 hypothetical people and asking them to rate the QoL of each person profile on a visual analogue scale. Regression analysis is then used to derive the weights for the five cues. Scores obtained using this method have been found to be responsive to change (Murrell et al., 1999).

However, as the judgement analysis method of item weighting is very time-consuming, the SEIQoL- DW, or direct weighting method was developed to provide a more practical tool. This involves weighting each of the nominated domains by

representing the relative importance of each area on a sectogram (a round disc with coloured segments like a pie chart) that can be adjusted manually so that the more important domains receive a larger proportion of the disc. Scores obtained using the direct weighting method have been found to relate closely to global ratings of QoL and are more sensitive to change than the SF-36 (Murrell et al., 1999). The SEIQOL-DW takes between 5 and 10 minutes to administer and has been recommended for use in large scale clinical trials as an adjunct to health status measures (Hickey et al., 1996).

2.4.2 The Patient Generated Index (PGI)

The Patient Generated Index (PGI) is an individualised measure of QoL based on Calman's definition (Calman, 1984) and the idea that by narrowing the gap between a patient's current state and their hopes and expectations there will be a consequent improvement in QoL (Ruta et al., 1994).

Completion of the PGI is similar to the SEIQoL in that it involves patients listing the five most important areas of their lives that are affected by their condition and rating how badly affected they are in each of the five areas. In a more recent version of the PGI two other categories are also rated. One of the categories refers to 'all other aspects of their life not already mentioned' and the other to 'areas affected by all other health problems' (other than the one already mentioned) (www.dundee.ac.uk/epidemiology/PGI). The final stage involves patients "imagining" they can spend 60 "points" (or 14 points in the updated version) in order to improve one or more of these areas. Point allocation is used to represent the relative importance that would be placed by the patient on an improvement in each of the areas. The final PGI score is designed to represent the extent to which reality falls

short of expectation in those areas of life in which the patient would most value an improvement. The individualised nature of QoL means that the PGI enables the generation of high-level activities (e.g. skiing, jogging, squash) compared to the physical functioning scale of the SF-36 which covers lower level physical activities mainly related to everyday activities (e.g. lifting heavy groceries, bathing and dressing).

The PGI is different from the SEIQoL in that it elicits the five most important areas affected by the patient's medical condition, rather than the five most important areas of life in general. This means that the particular influence of the patients condition on QoL should be highlighted and thereby make the measure more sensitive than the SEIQoL to treatment effects (Garratt et al., 1996). Nonetheless, the PGI has been recommended for use only as an adjunct to widely used generic questionnaires, such as the SF-36 following difficulties experienced in using the direct weighting system (point spending) and low response rates to a postal survey version of the scale (Macduff and Russell, 1998).

2.4.3 The Repertory Grid

The repertory grid method has been adopted as an individualised QoL measure (Thunedborg et al., 1993). This involves individuals' comparing their current self-image, "you as you are now", to a series of states including, "you as you were before treatment" and "you as you wish you were". Respondents are asked to list personally relevant issues within a set of predefined life domains (e.g. physical, social) and any other 'constructs' of importance and then rate these along with a set of predefined constructs.

Unfortunately the assessment is interviewer administered and time consuming, taking between 60 and 90 minutes to complete. It also requires the respondent to have a reasonably high level of verbal and cognitive ability thereby limiting its wider application.

2.4.4 Disease-specific Individualised Questionnaires

A number of individualised disease specific measures have also been adopted. This includes partially individualised measures for lung disease (Guyatt et al., 1987) the Idiographic Functional Status Assessment for AIDS patients (Rapkin, 1994) and the Audit for Diabetes Dependent Quality of Life (ADDQoL) (Bradley et al., 1999). These adopt a similar approach to the other individualised measures mentioned, requiring patients to generate activities or issues affected by their condition and/or rating their importance or subjective meaning.

2.5 Standardised measures of QoL that capture the subjective impact of disease

The two central properties of individualised QoL questionnaires are individually-generated domains and the importance ratings used to weight the domains. These are attractive properties considering the subjective and highly individual nature of QoL. However, it is not always practical to incorporate both of these attributes into questionnaires given the amount of time and resources required to administer the scales and problems associated with using the data they supply (e.g. assessing change over time when the issues nominated between assessments).

It is uncertain which of these properties is of greatest advantage (Leventhal and Coleman, 1997). Individually generated domains are more relevant to the patient than

domains imposed by a standardised measure. However, these domains may change over time and make it difficult to assess changes in QoL. The application of importance ratings to a range of universal or comprehensive life domains may be a more practical way of eliciting an individualised assessment of QoL domains (Bullinger, 1999; Carr and Higginson, 2001). This method has emerged over the last few years. These questionnaires do not ask patients to generate their own items, but obtain a better idea of individuals' QoL through patients' ratings of the importance of or satisfaction in the broad domains included in the measures (Wood-Dauphinee, 1999).

2.5.1 The Subjective Quality of Life Profile (SQLP)

The SQLP (Dazord et al., 1993), like the PGI, looks at the perceived distance between the individual's current state and their goals. The questionnaire provides standardised goals but respondents are questioned about: 1. the importance attributed to each goal; 2. tolerance of the distance between reality and goal state and; 3. their ability to cope with this distance. In delineating these issues the measure aims to provide an explanation behind individual evaluations of QoL.

2.5.2 International quality of life assessment: The WHOQOLS

The World Health Organisation Quality of Life Scale (WHOQOLS) was developed as the result of an international project set up to develop a questionnaire that would focus on positive aspects of people's lives and potential ways to strengthen them (WHOQOL Group, 1993; WHOQOL Group, 1995). The WHOQOL does not ask patients to generate life domains or goals, but attempts to evaluate life quality within the context of each individual's life by assessing satisfaction with particular life areas, or 'facets' of QoL. These facets are the same across the different versions of the

instrument, each comprising culture/country-specific items.

Satisfaction with life areas is believed to be more indicative of QoL than more objective measures of these areas (e.g. financial income) as it are easier to compare across culture or socio-economic status levels (Orley et al., 1998). For example with social activities, satisfaction can be compared regardless of the activity, setting, or frequency of participation.

Add-on modules for the assessment of QoL in specific populations (e.g. children, cancer patients, AIDS patients) have also been proposed. A shortened version of 26 items has been developed (WHOQOL-BREF; WHOQOL Group, 1998) and is recommended for use in clinical work, large-scale studies and clinical trials where only a brief assessment is possible.

2.6 Measurement of the QoL process

The aggregation of questionnaire scores into a profile or single index often masks the determinants of overall QoL. However, the process leading to QoL evaluations (Hyland, 1992; Leventhal and Coleman, 1997) may best be represented by a questionnaire or questionnaires designed to capture aspects of each level of the evaluative process (Fries and Singh, 1996; Spilker and Revicki, 1996). This would involve separating out these levels of disease impact into symptom experience, treatment effects, the impact of symptoms and treatment within the context of the individual's life and an overall evaluation of QoL.

2.6.1 The 'Measure Yourself Medical Outcome Profile' (MYMOP)

One questionnaire that might be useful in uncovering the determinants that underlie QoL is the Measure Yourself Medical Outcome Profile (MYMOP) (Paterson, 1996;

Paterson and Britten, 2000). Designed for use in general practice, this measure provides a problem-specific profile of scores. It requires patients to generate one or two symptoms of concern and to rate these for severity. It also elicits one activity of daily living affected by the symptom(s) and the degree of difficulty associated with this activity. The final question asks patients to rate their general well-being.

It has been argued that the MYMOP is more a measure of symptom impact than of QoL (Jenkinson, 1996). However, the questionnaire is not presented exclusively as a QoL measure but rather as a patient generated measure of disease impact. As the MYMOP looks at both the potential determinants of QoL and QoL itself as suggested in QoL-as-process accounts (Hyland, 1992; Leventhal and Coleman, 1997), it presents a guiding framework for new instruments.

2.7 Ethical Concerns in QoL Assessment

2.7.1 Interference with QoL.

The first ethical concern is that assessing QoL, particularly through utility methods such as the time trade off, or the standard gamble technique, could interfere with patients' QoL (Joyce, 1993). Asking patients to make trade-offs, for example between life expectancy and QoL could cause upset to severely ill patients, particularly undesirable if the research is to be of no direct benefit to these patients.

QoL self-report measures may also threaten the adaptive suppression used by many patients to protect themselves from negative feelings associated with their illness and its consequences (Cella, 1995).

In terms of the research process, unrelenting endeavours to recruit patients or ensure

the completion of forms may also be detrimental to QoL (Fraser, 1993).

2.7.2 Raising Expectations.

QoL questionnaires and the specific questions incorporated in these instruments may raise patients' expectations. Simply mentioning a QoL study or a QoL questionnaire may lead patients to believe that the assessment will lead to an intervention that will improve their life quality. Similarly, asking questions about medical treatment may introduce false hope in patients living with conditions for which there is no available treatment.

2.7.3 Confidentiality/ Privacy.

A number of the questions included in QoL questionnaires may be perceived as threatening, particularly questions about personal issues such as emotional state, body image and sexual activity (Smith, 1999). Patients may not wish to divulge this information to a stranger and may see it as immaterial to their medical care.

2.7.4 Ensuring the ethical investigation of QoL

Despite these concerns, avoiding asking questions about the effect of illness and treatment upon QoL could lead to the application of unnecessary or inappropriate treatment (Cella, 1995). However, it is important to consider the ethical implications and to ensure careful selection of questionnaires and strict confidentiality of responses before embarking on a QoL study.

2.8 Developing QoL Instruments.

Good QoL questionnaires tend to result from a rigorous development process involving item selection, item reduction, design of questionnaire format, pre-testing, and validation (e.g. testing for reliability, validity and responsiveness). Numerous different methods have been adopted in the development of QoL questionnaires. For example the Sickness Impact Profile (SIP) (Bergner et al., 1981), was developed on the basis of a literature review and the reports of health professionals as well as healthy and ill laypersons. The WHOQOL utilised expert review, focus groups, expert and lay question writing panels, development piloting and field testing (WHOQOL group, 1995).

Until recently, expert opinion has been used routinely in developing the content of QoL questionnaires. However, this has increasingly been abandoned and questionnaire items are now more commonly generated from patients' reports. This makes sense as QoL measures aim to identify patients' perceptions of QoL and doctors perceptions may not take important issues into account, particularly those of a less medical nature. QoL questionnaires are now more commonly developed using information about patients' experiences elicited using qualitative interviews and focus groups.

2.9 Quality of life in Neuromuscular Disease

Neuromuscular diseases (NMDs) are chronic progressive conditions that have numerous implications for QoL. There are few treatments available for NMD despite ongoing efforts to develop new treatments and use existing drugs in the hope that they will confer beneficial effects.

The treatment of Polymyositis (PM) and Dermatomyositis (DM) involves the use of steroids or other immunosuppressive drugs and recovery has been shown to be more common following the introduction of these treatments (Brain, 1985). However, there are no clear guidelines to the optimal dose or duration of treatment (Lane et al, 1989), and few therapies have been shown to be effective in randomized clinical trials (Miller et al., 1992; Dalakas et al., 1993). Other worthwhile interventions include physiotherapy and hydrotherapy as well as assistive devices such as wheelchairs, foot splints, respiratory support and aids in the home. The use of validated QoL instruments could better document the effectiveness of these interventions. The side effects associated with drugs currently used in certain muscle conditions such as corticosteroids in PM and DM further demonstrates the importance of investigating QoL in NMD.

In the absence of disease specific measures for NMD, researchers have often used the 'best measure' available. However, these generic measures may not always be suited to the investigator's purpose.

Generic measures including the SF-36 (Ware and Sherbourne, 1992; Brazier et al., 1992), the NHP (Hunt et al., 1985) and the SIP (Bergner et al., 1981) have been used in studies with NMD patients (Bach et al., 1991; Pehrsson et al., 1994; Ahlstrom and Gunnarsson, 1996; Ahlstrom and Sjoden, 1996; Ahlstrom et al., 1994; Drouet et al., 1996; Alexanderson et al., 1999; Chung et al., 2001). A disease-specific measure developed for use in rheumatoid arthritis, the revised Arthritis Impact Measurement Scales (AIMS2) (Meenan et al., 1992) has also been used in myositis patients (Drouet et al., 1996).

A number of these studies have shown NMD to have an influence on certain domains of QoL as assessed by the questionnaires used (Ahlstrom et al., 1994; Ahlstrom and Gunnarsson, 1996; Drouet et al., 1996; Chung et al., 2001). However, as these measures are not derived from patients' experiences of NMD they may neglect issues that are of considerable importance and conversely incorporate superfluous items. This means that they are unlikely to provide an accurate picture of QoL in muscle disease and may therefore also lack sensitivity to change.

Generic measures have also demonstrated unexpectedly high levels of life satisfaction in NMD patients with severe functional disability (Bach et al., 1991; Pehrsson et al., 1994).

There are two possible reasons behind the high levels of life satisfaction reported by NMD patients. The first is that questionnaires used in these studies might not be appropriate for use with patient populations or purposes divergent from those for which the questionnaire was intended. Therefore, the questionnaire items, their scaling and their wording may not be suitable for use with NMD patients.

The second possible reason for the high levels of reported QoL is the ability of individuals to adapt to their situation through changing their goals and expectations (Taylor and Brown, 1988; Lundqvist, 1991; Allison, 1997; Muldoon et al., 1998; Cassileth et al., 1984; Breetvelt and van Dam, 1991). These findings of high QoL levels have been mirrored in studies of other chronically ill or disabled patients (Cassileth et al., 1984; Burckhardt et al., 1989; Lundqvist, 1991). It may be that this process of adaptation results in QoL levels comparable to those of 'healthy' individuals making it difficult for QoL measures to detect any change in health (Allison, 1997; Breetvelt and van Dam, 1991; Sprangers and Schwartz, 1999; Gibbons, 1999; Albrecht and Devlinger, 1999).

These findings indicate the limitations in using generic questionnaires for patients with NMD. Muscle conditions are chronic and progressive and therefore it may be more appropriate to adopt disease-specific measures, widely heralded for their sensitivity to change (Patrick and Deyo, 1989; Sprangers and Aronson, 1991; Garratt et al., 1993).

The aim behind this study was therefore to develop a measure of QoL specific to adult NMD that would represent the effects of NMD as experienced by different individuals.

2.10 Hypothesis

Currently used measures of outcome in NMD encompass clinical measures such as muscle strength and biological indicators such as Creatine Kinase (CK) level. Although such measures provide information that is of clinical importance, they overlook individual circumstances and life context and therefore do not bear direct relevance to patients.

QoL questionnaires are used in clinical trials to represent patients' experience and the use of generic QoL questionnaires in a number of NMD trials has demonstrated increasing awareness about the importance of patients' point of view. However, these generic scales do not capture all the issues that are relevant to NMD patients and conversely incorporate superfluous issues. This means that such questionnaires are unlikely to detect clinically important change in patients with NMD.

The INQoL was therefore developed as a patient-centred measure that would capture the diversity of patients' experiences. The questionnaire asks each patient to rate their difficulties and the importance of the difficulties caused by specific symptoms. Patients are also asked to rate difficulties in specific life areas, their satisfaction with these areas of life and the importance of disease impact upon these areas.

It is hypothesised that this new disease-specific QoL questionnaire will be a more accurate indicator of QoL in patients with NMD than the measures currently available. As such, it should also be more sensitive to clinically important change and will be appropriate for use as a clinical tool, highlighting issues that may be amenable to intervention and helping monitor patients' progress.

2.10.1 Main hypothesis

There is a need for a neuromuscular disease-specific QoL questionnaire that is based on theories of QoL which centre around the idea that QoL is determined an individual's perception of the distance between their current state and the state to which they aspire. The individual's life context and disposition influence this perception. Such a tool should enable patients to representatively convey the influence of NMD upon their lives. This measure should also be more acceptable to patients than generic measures that are less likely to adequately represent NMD patients' QoL. Such a measure will also be useful in clinical practice providing a summary of QoL that can be used to highlight individual concerns and monitor progress over time.

2.11 Aims and Objectives

This thesis describes the development of a disease-specific QoL questionnaire for use in NMD. This patient-centred questionnaire will be used as a measure of outcome in clinical trials and as a clinical tool.

The objectives of the project were:

1. To explore what it is like to live with NMD with a view to designing a questionnaire based on patients' reported experiences. This was done through:

a. In-depth semi-structured interviews with NMD patients

b. A postal survey providing quantitative and qualitative data on the impact of NMD

2. To develop an individualised QoL measure specific to NMD for use as :

a. A measure of outcome in clinical trials

b. A clinical tool in muscle clinic consultations

3. To assess the clinimetric properties of the questionnaire, including its:

a. Validity

b. Reliability

c. Responsiveness to change

4. To evaluate the questionnaire in a clinical setting for its:

a. Acceptability to doctors and to patients

b. Clinical utility/usefulness

2.12 The Structure of the Thesis

This project involved several phases including three separate studies, two of which determined the content of the new questionnaire (qualitative interviews and postal survey) and the third of which involved testing the questionnaire resulting from these exploratory studies.

In order to ensure a clear account of the development and validation of the INQoL the thesis is structured to present the background, preparation, implementation and findings of the individual studies separately and chronologically (table 2.1, p.68). Chapter 5 describes principles of questionnaire design and comprises a section of its own. It precedes the postal survey and INQoL design and validation chapters as the issues described were relevant in constructing the survey instrument and QoL questionnaire.

Table 2.1: Thesis structure

Introduction
Chapter 1: Review of QoL literature
Chapter 2: Review of existing QoL questionnaires and the assessment of QoL in NMD

Qualitative interview study
Chapter 3: Description of qualitative research and interviewing
Chapter 4: Qualitative interview study introduction, methods, results and discussion

Questionnaire design issues
Chapter 5: Constructing questionnaires

Postal Survey
Chapter 6: Description of general postal survey methodology
Chapter 7: Postal survey introduction, methods, results and discussion

Design and validation of the INQoL
Chapter 8: Description of QoL scale design methodology & the construction of the INQoL
Chapter 9: Description of the clinimetric properties of QoL instruments and their evaluation
Chapter 10: Clinimetric evaluation of the INQoL (methods)
Chapter 11: Clinimetric evaluation of the INQoL (results and discussion)
Chapter 12: Clinical utility study

General Discussion and Conclusions
Chapter 13: Discussions
Chapter 14: Conclusions

CHAPTER III

QUALITATIVE INTERVIEWING AND QUALITATIVE DATA ANALYSIS:

THE FIRST STAGE IN THE DEVELOPMENT OF A QUALITY OF LIFE QUESTIONNAIRE

Chapter 3: Qualitative Interviewing and Qualitative Data Analysis: The first stage in the development of a quality of life questionnaire

The first stage in the development of the disease-specific quality-of-life questionnaire involved exploring neuromuscular (NMD) patients' experience of their condition.

QoL in NMD has previously received little attention. The aim of the initial phase of the study was therefore to obtain a representative account of the experiences of NMD patients through qualitative interviewing and data analysis. This chapter will provide an outline of qualitative research and its methods.

3.1 What is qualitative research?

Qualitative research takes the form of exploratory investigation that focuses upon 'naturally occurring, ordinary events in natural settings' (Miles and Huberman, 1994). In investigating the experiences of individuals, qualitative research asks the questions 'what?, 'why?' and 'how?' enabling in-depth exploration of a particular phenomenon or experience. This contrasts with the question asked in quantitative research, 'how much?' which should be asked once the issue under study is better understood (Pope and Mays, 1995).

3.2 Why use qualitative research?

Qualitative research is useful in investigating issues about which little is known, or in which there has been little previous research. This makes it perfect for exploring the effects of NMD considering that there are no existing disease-specific measures of QoL for these conditions.

Most existing scales are based upon the opinions of experts rather than upon those of patients, or upon existing QoL scales such as the SF36 or the Sickness Impact Profile. However, these questionnaires do not always accurately represent the patient's point of view (Carr, 2001). The use of a qualitative methodology was thought likely to result in a more accurate representation of patients' experiences, for use as the basis for a patient-centred questionnaire.

One of the main advantages of using qualitative research is that it requires only small numbers of participants, yet allows issues to be explored in great depth and results in rich and detailed data.

Qualitative research can also be a useful precursor to quantitative research (Pope and Mays, 1995), giving an indication of the areas to probe further for quantitative information. This can be useful in the development of QoL scales as quantitative methods can be used to generate information about the relevance and importance of potential scale items. This type of methodology has already been used in the development of questionnaires (Bryman, 1988) including QoL scales (e.g. Hunt et al., 1985).

3.3 Theoretical frameworks used in qualitative research

Among the remarkable array of approaches to qualitative analysis are the five main traditions of biography, ethnography, case study, grounded theory and phenomenology (Cresswell, 1998). These traditions are each geared towards a specific field of research or have a specific aim.

3.3.1 Biography

A biography is the study of an individual and his/her experiences and has been defined in the *Oxford English Dictionary* as 'a written record of the life of an individual'. Biographical studies tend to involve interviews and the analysis of documents (e.g. letters, diaries) about the individual (Smith, 1995).

3.3.2 Ethnography

An ethnography is the description and interpretation of a culture, social group or system. It involves the researcher in an extended observation of the group often through living alongside the people in their everyday lives or interviewing members of the group. This allows the examination of the group's observable behaviours, customs and ways of life in order to achieve a better understanding of their behaviour, language and communication (Cresswell, 1998).

3.3.3 Case Study

A case study involves investigating single or multiple cases over time though detailed data collection involving many different sources of information (e.g. observations, interviews, and documents). It is not a methodological choice but more of a choice of the object to be studied (Stake, 1995).

The case may be an individual or an event or even an activity situated within its usual setting. It is most commonly heard of in relation to medical case histories in which a picture is built up of an individual patient with regard to a particular issue (e.g. condition or complaint). This may involve observation and interviews, examination of medical reports and investigations and consultation with health professionals who have worked with the patients.

3.3.4 Grounded theory

Grounded theory has been described as a general methodology for developing a theory that is grounded in systematically gathered and analysed data (Strauss and Corbin, 1995). It involves the researcher in interviews and visits to the field of interest in order to investigate actions and reactions to a phenomenon in a social context (Cresswell, 1998). Grounded theory analysis entails developing and interrelating categories of information in order to form theories and hypotheses about the topic of interest (Strauss and Corbin, 1995).

3.3.5 Phenomenology

Founded upon the philosophical writings of Husserl (Cresswell, 1998; Sokolowski, 2000), phenomenology is the 'study of lived experiences and the way we understand those experiences to develop a worldview' (Marshall and Rossman, 1999). It is used to investigate a specific experience or phenomenon in an attempt to see things from the others' point of view (Taylor and Bogdan, 1984).

Phenomenologists' interpretation of qualitative data is built on the assumption of a stable relationship between 'outward appearance' (what someone says) and 'inner consciousness' (underlying feelings and psychological constructs) (Giorgi, 1995). This assumption is central to the development of an understanding of the experience of a particular phenomenon (Cresswell, 1998). Phenomenological analysis will be described in section 3.6.3.

3.4 Which approach to Adopt?

Researchers holding more traditional approaches to qualitative research (Strauss, 1987) advocate the application of specific and unadulterated methodology from one of the qualitative traditions. This is believed to maximise the validity and reliability of qualitative research. However, it may not be appropriate to impose exact interpretative structures onto social and psychological phenomena as life experiences are unlikely to fit into such a strict analytic framework. A more eclectic approach to qualitative analysis has therefore been advocated (Seale, 1999).

Nonetheless, it is helpful to base qualitative analysis around one particular tradition in order to guide the study and keep the complicated process of data analysis as straightforward as possible.

As the aim of this study was to describe and better understand individuals' experience of NMD, a phenomenological position was adopted with key elements of phenomenological analysis applied to the interview data (Chapter 4).

3.5 Methods used in Qualitative Research

Participant observation, in-depth or semi-structured interviewing, and focus groups are just some of the techniques used in qualitative research (see Miles and Huberman, 1994, and Denzin and Lincoln, 1994; for a full list of the available techniques).

The choice of method depends largely upon the issue under study and upon the resources and time available to the researcher. For example, focus groups (group interviews) are ideal for the researcher conducting an inexpensive study to explore a

socially relevant phenomenon occurring naturally in a particular group (e.g. attitudes or cultural practices).

For the purposes of this study it was considered most appropriate to use semi-structured interviewing. Although this is more time consuming than focus groups, requiring a greater number of interviews, it allows a more thorough exploration of individuals' experience. It was felt that this would provide the best basis for the questionnaire.

3.5.1 Qualitative interviewing

Qualitative or 'in-depth' interviewing is frequently adopted in health services research. These studies are logistically less onerous to organise and conduct than studies involving people in their natural environment, such as participant observation, that require the researcher to be immersed in the life of a group or community for a prolonged period of time (Punch, 1995). Qualitative interviews glean rich data and are highly flexible in exploring areas of interest.

Good questions in qualitative interviews are open-ended, non-leading, sensitive, and clear to the interviewee (Britten, 1995). Unlike the structured interview, questions are adapted to suit the interviewee's style of speech. Questions are posed within the natural flow of the interview and interviewees are encouraged to clarify and elaborate upon what they disclose (Pope and Mays, 1995).

Researchers conducting interview studies aim to establish rapport and create a relaxed atmosphere conducive to the disclosure of potentially sensitive and personal information. It is therefore important for the interviewer to pay attention, be non-

judgemental, to let the participant talk, and be sensitive to his or her feelings throughout the interview (Taylor and Bogdan, 1984).

3.5.2 Semi-structured interviewing

In phenomenological interviewing the researcher commonly has an idea of the issues that might be important to look at. In this situation is makes sense for researchers to conduct semi-structured rather than unstructured interviews in order to maximise the relevance of the data. Semi-structured interviews are guided by the use of an interview schedule (Britten, 1995).

3.5.2.1 Interview schedules

Interview schedules are useful in studies that aim to delve into particular areas of interest and are helpful in the preparatory phase of interview studies, compelling the researcher to consider all the issues to be covered. This helps prepare the researcher for any sensitive issues that might arise and gives him/her the opportunity to devise appropriate question wording and any additional questions that may be necessary.

Interview schedules tend to open with a broad, general question such as " tell me about ...." or "what is your experience of .....". This allows the interviewee to set the ball rolling and share his/her own account of the experience. The interviewer can then guide the interview depending upon what the interviewee is interested in talking about and what s/he wishes to cover.

3.5.3 Audio-taping interviews

Tape-recording allows the researcher to obtain a full, representative account of the interview. It frees the researcher from writing notes, which can be disruptive to the

process and may result in loss of information. This also helps the researcher to establish rapport with the interviewee, to concentrate on the flow of the interview and formulate appropriate questions to ask next.

Although some participants might experience 'stage fright' and not feel able to talk freely during the interview, the presence of the tape recorder should not greatly alter the participant's responses. The presence of an attentive researcher is likely to negate any influence a tape recorder may have.

3.6 Issues in qualitative research

3.6.1 Sampling

In qualitative research sample sizes are not determined by specific rules. Rather than aim for statistical representativeness, the number of participants selected tends to be based on the depth and duration of the interviews and the nature of the sample (Mays and Pope, 1995).

Ten participants from a fairly homogenous sample is generally accepted as a reasonable number to obtain in a qualitative interview study (Greenhalgh, 1999; Riley, 1996). 30 to 50 participants are more common when there are subgroups of interest within the sample (Morse, 1994).

The sampling procedures adopted tend to be purposive (Miles and Huberman, 1994), aiming to obtain a reasonable cross-section of the individuals experiencing the phenomenon of interest. This allows the experiences of subgroups within the sample to be illustrated and facilitates comparisons between the groups. A purposive sampling strategy was adopted in this study in order to represent the experiences of

three subgroups of patients and enable the detection of any differences between these diagnostic groups.

3.6.2 Analysis

There are an abundant array of texts available on qualitative analysis (e.g. Miles and Huberman, 1994; Coffey and Atkinson, 1996; Denzin and Lincoln, 1994; Strauss, 1987; Riley, 1996). Some focus upon a specific school of qualitative research (e.g. Strauss, 1987; Giorgi, 1995) whereas others advocate a more eclectic approach to qualitative analysis (Seale, 1999; Miles and Huberman, 1994). Despite this, the process of analysis is similar across the different traditions. They all involve a cyclical rather than a linear process with the various stages of analysis feeding back onto one another.

3.6.2.1 Description

The analysis of qualitative interviews starts off with reading and re-reading the transcripts to ensure complete familiarity with the data. The researcher then notes down any striking or interesting patterns and themes that come forth and reflects upon what seems to be happening in respondents' accounts.

3.6.2.2 Organisation

This stage involves organising the data in a way that helps to answer the research question. Codes are applied to each theme and used to pick out occurrences of the themes from the transcripts. Each coded segment is cut out from the transcripts (this can be done using a word processor) and pasted into files together with all the occurrences of each particular theme. This simplification of the data into manageable segments eases the retrieval of categorised data (Coffey and Atkinson, 1996), which

can be further simplified by indexing the occurrences and location of themes (Smith, 1995).

3.6.2.3 Connecting segments and interpretations

In this next stage themes that have emerged from the ongoing analysis are linked up and subsumed into broader categories. This is done through comparing and contrasting themes as well as identifying patterns and new meanings in the analysis. Following this process, the categorisation system is checked against all the other transcripts to ensure comprehensive coverage of themes and meanings in the data.

3.6.2.4 Corroboration and justification

This next stage involves checking the analysis to ensure its credibility and increase confidence in the results. One way this can be done is to look for unexpected themes in the data in order to elaborate on emerging themes and dispute inappropriate interpretations. This leads to more refined and detailed descriptive categories. Counting techniques (Miles and Huberman, 1994; Riley, 1996) may also be used to give an idea of the prevalence of themes (Smith, 1995). However, the use of this technique to reduce data into a simplified quantitative representation is discouraged (Miles and Huberman, 1994) and should only be used to verify aspects of the data.

The use of a second categoriser is one of the many strategies that can be used to triangulate qualitative data. 'Triangulation' is the main method used in qualitative research to validate findings. This involves using several methods at once to measure the same phenomenon with a view to cancelling out any biases that result from the other methods (Webb et al, 1966 cf. Seale, 1999). The independent measures that can be used include different data sources (e.g. patients and their doctors), methods (e.g.

interviews and fieldwork), different researchers, or different types of data (e.g. qualitative and quantitative) (Miles and Huberman, 1994).

The second categoriser analyses interview transcripts using the categories emerging from the original researcher's analysis. This helps to ensure that the coding scheme is comprehensive and may also throw light upon any ambiguities in the analysis or difficulties in the application of categories (Riley, 1996).

3.6.2.5 Reporting Findings

The process of analysis culminates in writing up the findings of the investigation. Writing up further influences the interpretation of the data and often leads to a more complete and integrated interpretation.

3.6.3 Phenomenological analysis

The stages delineated in the previous section are also utilised by phenomenological researchers. However, there are a number of components particular to this tradition (Giorgi, 1995; Cresswell, 1998).

3.6.3.1 Epoche

'Epoche' is the term used to refer to the first stage in phenomenological analysis. This involves the researcher giving a full description of his or her experience of the phenomenon, 'bracketing' previous any experiences and preconceptions before commencing analysis. This allows the 'phenomenon' as described by the participants to be interpreted with as little bias as possible.

3.6.3.2 Horizonalisation

Horizonalisation in phenomenological analysis constitutes the 'organisation' phase referred to in the previous section. This process involves finding statements in the transcripts about individuals' experience of the subject of interest. These statements are listed and any repetition or overlapping minimised.

3.6.3.3 Clustering of statements into 'meaning units'

This stage comes under the 'connecting themes and interpretations' phase delineated earlier. It involves clustering together statements that represent the emerging descriptive themes, along with examples of these statements from the transcripts.

3.6.3.4 Reflection and Description

This final phase reflects elements of the third (connecting themes and interpretations) and fourth stages (corroboration and justification) of qualitative analysis as described previously.

The researcher reflects upon the description of the experience using intuition and imagination to search for and provide all possible meanings and perspectives of the data. By looking at the phenomenon from these different perspectives the researcher can construct a description of the experiences of those participating in the study.

These stages should result in a report that increases the readers understanding of what it is like to experience the phenomenon of interest.

3.7 Using Qualitative and Quantitative Research as complementary exploratory research methods

As expressed by Miles and Huberman (1994) 'numbers and words are both needed if we are to understand the world'. Indeed, qualitative and quantitative research can be used to confirm or corroborate each other via 'triangulation', resulting in an ultimately more detailed investigation (Miles and Huberman, 1994). For example, surveys can be used to corroborate and supplement qualitative data, reaching those not represented in the original investigation, for example housebound individuals unable to travel to attend a qualitative interview. They can also provide a measure of the prevalence of the issues reported in qualitative studies, a valuable statistic when selecting items for inclusion in a questionnaire. A survey was therefore adopted in this study to corroborate the representativeness of the data from the qualitative study (Chapter 7).

3.8 Conclusions

Qualitative research is useful in exploring experiences about which little is known, making it ideal for the purposes of this study. Semi-structured interviews were adopted and a predominantly phenomenological analytic technique used to facilitate a thorough exploration of the experiences of individuals with NMD.

CHAPTER IV

EXPLORATION OF PATIENTS' PERCEPTIONS OF THE IMPACT OF NEUROMUSCULAR DISEASE: A QUALITATIVE INTERVIEW STUDY.

Chapter 4

Exploration of Patients' Perceptions of the Impact of Neuromuscular Disease: A Qualitative Interview Study.

4.1 Introduction

Conducting exploratory qualitative work has been useful in previous studies of individuals with chronic disease and disability (Viemero and Krause, 1998; Albrecht and Devlieger, 1999) and is also useful in the construction of questionnaire items (Bryman, 1988). Such methods have been used to generate the life domains covered in other QoL questionnaires (e.g. Hunt et al, 1985).

As little is known about the impact of NMD, qualitative interviews were used to explore patients' experiences. It was felt that a QoL questionnaire developed from patients' personal experiences would elicit a more representative picture of disease impact in individual patients than the measures currently in use. It should also be more sensitive to change than questionnaires derived from the opinions and conjectures of health professionals.

4.2 Methods

In-depth interviews were conducted to explore patients' experiences and perceptions of the effects of NMD upon their lives.

4.2.1 Patients

a. Patients involved in the project as a whole

Muscle diseases involve a combination of weakness, fatigue, stiffness or cramps which is usually progressive. Clinical diagnosis of a specific muscle disease tends to be based on the age of onset, the mode of inheritance (where there is a family history) the distribution of weakness (e.g. whether proximal or distal) and the presence or absence of associated features such as pseudohypertrophy in muscular dystrophies, or myotonia in myotonic dystrophy (Brain, 1985).

For the purposes of the study, three groups of patients were sampled as follows;

1) patients with a congenital, slowly progressive NMD (CSP),
2) patients with an acquired, relapsing, remitting NMD (ARR), and
3) patients with an acquired, slowly progressive NMD (ASP).

Those in the CSP group included patients with non-Duchenne muscular dystrophies such as Becker's muscular dystrophy and limb-girdle muscular dystrophy (LGMD). A number of patients with genetic muscle conditions that are not always apparent at birth were also included. These included patients with facio-scapulo-humeral muscular dystrophy (FSH-MD) and myotonic dystrophy. Many of these conditions can occur in mild or severe forms, some of which may be associated with a loss of ambulation (e.g. in second or third decades for LGMD) (Marsden and Fowler, 1998).

Patients with Duchenne muscular dystrophy (DMD) were excluded from the study as these patients often die before the age of 20 (Marsden and Fowler, 1998). They are therefore likely to face different QoL issues than patients with other muscle conditions

(e.g. different approach to care and issues of death and dying).

Those in the two 'acquired muscle condition' groups were made up of patients with either 'relapsing remitting' (e.g. polymyositis (PM) and dermatomyositis (DM)) or 'slowly progressive' (e.g. inclusion-body myositis (IBM)) disease. These conditions are primary inflammatory muscle diseases but DM can also involve other organs including the cutaneous, gastrointestinal, pulmonary, and cardiac systems (Miller et al, 2001), while PM has only occasional extra-muscular involvement (e.g. pulmonary) and IBM has no systemic involvement. PM and DM are amenable to treatment with steroids or other immunosuppressive drugs but they can go through periods of remission and relapse as the treatment is altered. IBM tends to have a later onset than PM and DM, being more commonly diagnosed in patients over 50 years of age. It has thus far been resistant to treatment and so pursues a progressive course (Griggs and Rose, 1998).

Patients were split into these groups as it was predicted that there might be differences in experience depending upon individual characteristics of the different conditions. For example, patients with congenital conditions are likely to have lived with the effects of their condition from a young age. On the other hand, patients in the ARR group are likely to have an unpredictable disease course. Finally, in the ASP group the onset of disease tends to be later in life. It was predicted that these patients might be faced with problems and ways of handling their condition that differ from those in whose condition starts at a younger age.

b. Patients involved in the qualitative interview study

41 patients took part in the interview study. A purposeful sampling strategy was used to select a minimum of ten patients from three diagnostic groups in order to enhance the likelihood of obtaining a representative picture of disease impact (Greenhalgh, 1999; Miles and Huberman, 1994; Smith, 1995; Crabtree and Miller, 1999). 15 male and 26 female patients ranging from 20 to 80 years of age were interviewed (mean= 49 years) (see Table 4.1 for full description of patients). These included 21 patients with a congenital, slowly progressive NMD; 10 patients with an acquired, relapsing, remitting NMD; and 10 patients with an acquired, slowly progressive NMD.

Table 4.1: Patients involved in interview study

	M:F ratio		Age range
CSP group	3:4	Facio-scapulo-humeral muscular dystrophy (2M, 1F) Limb-girdle muscular dystrophy (2F) Myoshi distal myopathy (1F) Becker's muscular dystrophy (1M) Muscular dystrophy (1M, 2F) Myotonia congenita (1F) Myotonic dystrophy (4M, 5F) Mitochondrial myopathy (1M)	20-75 years
ARR group	1:4	Polymyositis (6 F) Dermatomyositis (2 M, 2F)	43-80 years
ASP group	2:3	Inclusion-body myositis (4M, 6F)	20-67 years

4.2.2 Data Collection

In-depth, tape-recorded interviews were conducted in a quiet room in the outpatient clinic. Each patient was informed of the rationale behind the study and about the interview process before the interview commenced. Three patients requested to be accompanied by their spouse and one by her daughter. This was granted, as although patients' responses might be influenced by the presence of a close family member, it was

considered more important for patients to feel confident and at ease.

Patients were encouraged to explain in as much detail as they could, how their muscle condition had influenced their lives. Open, non-leading questions guided by an interview schedule (Table 4.2) enabled patients to respond in their own words.

Table 4.2: Interview guide for qualitative interviews with NMD patients

Interview guide

- Tell me about your condition and the effect you feel it has had upon your life.
- How are your daily activities affected by your condition?

  If not mentioned/volunteered, probe for :

  General activities (e.g. getting around, household tasks)

  Employment (working practices, relationships at work, financial concerns)
- Tell me about your social/leisure activities.

  Impact of NMD on these activities

  Friendships and social interaction
- How has your family life been affected by your condition?
- How has your condition affected how you feel other people see you?
- What effect has your muscle disease had upon your outlook on life?

The guide was constructed with the aim of encompassing all the life areas that could potentially be influenced by NMD. It was acknowledged that although the use of an interview guide might prompt issues reported, a semi-structured approach was more likely to ensure thorough exploration of patients' experiences (Britten, 1995; Riley, 1996; Smith, 1995). Expansion on reported experiences was encouraged by using phrases, such as "How did that make you feel?" and "Could you tell me more about that?" along with the use of prompting and pauses.

During the interviews it became evident that the line of questioning generated by the

interview guide was not always fruitful in exploring the experiences of patients with congenital conditions. Some patients found it difficult to explain their experience of NMD in terms of an "effect" or "effects", as they had not experienced a change in their physical condition. In many congenital patients no such change had occurred, or if it had, it was imperceptible to them given that they had lived with the physical effects of their condition from a young age. A modified interview guide (table 4.3) was used in 5 interviews to explore conceptual issues of disease impact in congenital patients, namely what it meant to them to be diagnosed and living with NMD.

Table 4.3: Modified interview guide for qualitative interviews with NMD patients

Modified interview guide
• How did you find out about your muscle condition?
• How are you affected by your muscle condition in your everyday life?
• How do you feel others see you?
• How does your muscle condition affect how you see yourself?
• Do you feel your muscle condition has stopped you from doing anything you would have done otherwise?
• Do you feel your muscle condition influences who you choose to spend your social time with?

The original interviews lasted between 21 minutes and 1 hour and 13 minutes and the 5 supplementary interviews lasted between 12 and 30 minutes. All interviews were transcribed verbatim, including details of intonation and non-verbal communication.

4.2.3 Analysis

Principles used in the management, organisation and analysis of qualitative data were adopted (Bryman, 1988; Marshall and Rossman, 1999; Cresswell, 1998; Giorgi, 1995; Miles and Huberman, 1994; Seale, 1999; Silverman, 1993; Smith, 1995). In order to

achieve an idea of what it is like to have NMD the theoretical approach of phenomenology (Smith, 1995; Giorgi, 1995) was considered to be the most appropriate analytic method. This was used to guide the analysis and facilitate the exploration of patients' perceptions and experiences.

Following repeated reading of the transcripts, underlying themes were extracted and clustered together into categories representing life domains influenced by NMD. A coding scheme was devised to represent the individual domain and subdomain categories and applied to the data (Appendix A). Modifications were made to the scheme during analysis to ensure consistent and comprehensive coding of the data. This was achieved through comparing and clustering life domains and the effects of NMD within these domains.

Finally, the comprehensiveness and validity of the coding scheme were further verified (Riley, 1996; Miles and Huberman, 1994) through inspection by a second researcher who independently applied the scheme to a sample of the interviews.

4.3 Results

The physical effects, their consequences, and the expected consequences of NMD had an important impact upon psychosocial aspects of patients' lives, including their emotions, self-image and perceived ability to fulfil their aspirations.

The broad life domains influenced by NMD will be described in the next section followed by a more detailed account of how NMD affects quality of life.

4.3.1 Life domains affected in NMD patients

The analysis of the interviews showed that NMD has a profound influence in patients' lives. (The numbers in brackets next to each excerpt indicate different patients).

> ....it's just there every minute of the day, everything you do (1)

A number of broad life domains (Table 4.4) reported by patients to have been affected by NMD emerged from the analysis. Most patients reported an impact in each of these life domains.

Table 4.4: Life domains reported in interviews with NMD patients

Symptoms	**Activities**	**Psychological domain**	**Social domain**
Physical symptoms	Daily activities	Emotions	Social interaction
	Leisure activities	Self perception	Relationships
	Employment/work	Perception of the future	Dependence

Many of the specific effects within these dimensions were unique to individual patients. These depended on the specific physical effects of their muscle condition, but also on individual characteristics and life situation. For example, reported effects upon relationships varied according to factors such as life stage and individual circumstance. This is demonstrated in the following quotations. The first is from an elderly gentleman diagnosed with Inclusion Body Myositis and the second from a 36-year-old woman with Polymyositis.

> I have grandchildren who want me to pick them up and I can't even lift a baby up, which is very

upsetting for me. They now say Granddad in the chair, I'm Granddad in the chair.....it upsets me. (2)

...I feel like he feels he has to stay.....I don't want him to stay just because I'm not well... And because it's a long going thing, does he feel trapped?..... I feel insecure, whereas I never did before, I just feel that maybe he feels sorry for me, and I don't want him to." (1)

4.3.2 Physical effects of NMD and its treatment

4.3.2.1 Symptoms

Some of the physical effects of muscle disease directly influenced patients' well-being. Fatigue, muscle weakness and pain were commonly reported.

> you're always very tired, lethargic, you know. You're always achy, you know in your muscles. If you try and do anything, your muscles tend to ache even more. It's quite demoralising in a way 'cos the more you do the worse you get... (3)

> If I lay in bed too long, I start to ache... I don't often sleep late 'cos when I'm awake I start to ache. So most days I'm up by four. (4)

Symptoms more specific to diagnosis were also reported. For example, a larger proportion of congenital NMD patients reported problems with vision and with speech.

4.3.2.2 Effects of Treatment

The side effects of drug treatment were important to those who had experienced adverse effects. Patients with Polymyositis and Dermatomyositis (in the acquired, relapsing, remitting subgroup of patients) reported side effects more commonly than patients with a congenital or an acquired, slowly progressive condition. This is because more drug treatments, notably steroids are used in these patients. Side effects took the form of

physical complications, effects upon mood, and changes in physical appearance brought about by steroid treatment. Some patients perceived their treatment to have caused as many problems as their muscle disease had done.

> I think really, the medication has done as much to wreck my life as the actual disease has done. Steroids particularly are quite awful to be on. (1)

The negative effects of weight gain caused by steroid treatment in patients with myositis were a particularly important feature in the interviews. Some patients also expressed their feelings about the potential consequences of steroid treatment, such as this patient with Dermatomyositis.

> I would like to come off the drugs eventually because I know that it's causing other problems by being on them, like osteoporosis. I had a bone scan just before Christmas and they said then that my bone density wasn't what it should be for my age and you think "well, if that's what it's like now, what is it going to be like in ten years time if I'm still taking the steroids?" (5)

4.3.3 Control & Independence

The amount of control and independence patients' had in their lives was important to their perception of NMD effects.

4.3.3.1 Control

Many of the negative consequences reported could be framed in terms of a loss of control. In many cases the loss of control described was of a physical nature, encapsulated in the frequent accounts of falling.

> I fall down in the street, crashing my head on the ground, and I find I can't lift myself up. So I have to rely on passers-by to help to lift me up, which is OK on a couple of occasions, but... (2)

These and other instances of physical loss of control were a source of extreme distress to many patients often representing a serious threat to their psychological or physical well-being.

> You're in a situation where you can't breathe, you've got food wedged in the throat, and the muscles have been weakened..... and so you've got this wad of food in the gullet and the damn thing won't go down and it won't come up and you can't cough it up, because it's not in the windpipe. It feels exactly as if you've got food choking you, stuck in the windpipe, but it's not, it's in the foodpipe, and so you're stuck there trying desperately to breathe and that can be very, very worrying. (6)

Symptoms and physical disability impinged upon the degree of control patients had over other aspects of their lives, notably their emotions.

> ...and with these disabilities you can't, sometimes you can't cope and sometimes you just explode, you know, you get very angry and uptight at the wrong times. (7)

Loss of control in the context of working practices and the implications of this for future job prospects led to feeling of helplessness.

> I mean we had to escort somebody out of the building the other day 'cos he was drunk, and I couldn't get hold of him, he just shook me off..... and our jobs are on the line... then I think ...'what am I supposed to do to get another job?' (8)

This kind of loss of control also had implications for fulfilling roles, such as the role of

employment.

> it feels a bit of a daunting prospect, leaving work. It doesn't feel right, does it? You're working, and not to be working, it doesn't quite feel right in my mind. (9)

One man described the psychological impact of his perceived loss of control in fulfilling his family role.

> I've been through a period quite recently where I was having different dreams, one after the other, where I was in a scenario that I couldn't look after my kids in a physical sense. I think it was obviously playing on my mind but especially because I'm one of these people that takes on everybody's problems..... It's always me that has to take control and sort everybody out and over the last six months I've really, sort of, lost the will to fight, if you like. (10)

4.3.3.2 Independence

Independence was an important issue in patients' reports. Dependence upon other people to perform certain tasks had a notable effect upon well-being. For some patients, dependence on other people influenced the amount of control they felt they had over their lives and the amount of freedom they could exercise. Independence was clearly important to patients' sense of identity and self-esteem with many striving to retain their independence. Some described their desire to retain as much normality in their lives and in how other people perceived them as possible.

> It's like with a can of coke and I'm having trouble, you'd feel the urge to take it off me and open it for me, you can understand that (laughs), 'cos you'd see I was in trouble... but I'll manage...That's the kind of thing I'm trying to get at. It's nice of them but it's annoying at the same time. I want them to be the same as they were before I found out I had this. (8)

Patients in the ASP group most commonly reported discontentment about their level of independence. On the whole these patients were older than the other patients with more of them living alone meaning that more of the help they required came from professionals and family members who could not always be available to help.

> [I have] to rely on being carried about. Whereas I just got in my car and I went. So, I can no longer make my own decisions about when I want to go, where I want to go...and so on. (11)

4.3.3.3 Maintaining Control and Independence

Efforts to regain or maintain control were reported by all patients. Patients commonly reported the measures they had taken and adaptations they had made to maintain control and as much freedom as possible. These adaptations included changing their accommodation, as described by one lady.

> I had a lot of building work done and I've arranged it, keeping in mind that there will be wheelchairs. I've made all my accommodation downstairs, and I have got two rooms upstairs if anyone lived in. I'm trying to keep the garden, so I could just have a man in to do the grass and the borders. (12)

Changes were also made in employment practices, such as cutting down the number of hours worked.

> I have recently cut my hours. I work more part-time now. Three and a half days a week, which I've found helps 'cos it was, I was working long days, sort of 8 'til 6 o' clock and, which is tiring. Get home and that was it, I just wanted to go to bed then. So cutting my hours has helped. (13)

Maintaining independence also necessitated more careful planning of activities.

I found I have to think a lot more about whatever I do. Plan a lot more about how I do things. I try not to let it stop me doing things.... and I have to think a bit more about how I am going to do them and who's going to go with me. I don't feel particularly safe on my own anywhere. (13)

4.3.4 Restrictions in Participation

The adaptations patients made to minimise the influence of their condition upon well-being enabled them to manage their lives and retain as much autonomy as possible. However, patients' avoidance of situations in which they might lose control restricted them in their activities. In many cases patients' social interaction and their personal relationships were influenced, which was detrimental to their emotional well-being and self-image.

I've been flat on my face in Soho Square in the early hours of the morning. I used to fall over everywhere I went. Something would trip me up or I'd lose my balance (*laughs*)... and to avoid those situations ... you just stop going and if you stop going you become quite a boring friend. (14)

Loss of control, and efforts to maintain control also had implications for carrying out activities and plans of choice.

Our idea, years ago, was to buy a little bar in Spain and live out there. So a few years ago we was looking at different bars and things ..... of course, I wasn't feeling great so we stopped looking and now I've got this, that's gone out of the window. (15)

Fulfilment of aspirations such as having children was also restricted, particularly in patients with a CSP condition.

I actually feel quite cheated that, even though I'm not going to have another baby, 'cos I'm not up to it..... if I did have, I couldn't look after it on my own, which wouldn't be fair.
I would've liked to, you know. (14)

Restrictions to participation were also experienced in activities such as employment and family activities

I went on a firefighting course back when I was a gas technician and ... I got to the stage where I couldn't lift up one of the fire extinguishers and fire it at the fire and they said, "you've got a problem haven't you?" and of course I had to tell them... I had to go and see one of British Gas's doctors..... and he said, "well, you've obviously got a problem, I don't know what it is. Do you?" and I explained it to him and he said, " I'm sorry", he said, " but we can't employ you any more".... and I'd done thirty-two years up 'til then and he said "well, you've got a very good record, we'll put you up to forty years and medically retire you" (16)

I'm just not so much fun because I don't rough and tumble with them. I mean, they like to climb trees and play chase and all that and I just can't do it and so it's just like, "Mummy's no fun" you know, they sort of have that feeling of, " oh Mummy can't do this and ...." you sort of think, "I wish I could, I wish I could just run around with you and play but I just can't. (17)

Barriers in the physical environment also hampered participation in social activities.

My friend down the road used to take me to the theatre. Well, last time it was such a fiasco, we had half the audience trying to get me up from the seat. So of course, I haven't been since. I can't go to the theatre, I can't to concerts, I just don't go out (18)

4.3.5 Effect upon relationships

The consequences of NMD also affected the relationships of many patients interviewed.

Of course it's not only stress on me it's stress on my wife as well because when I get uptight and angry I take it out on the closest one, which is my wife. (19)

This had further implications for patients' emotional well-being.

> it is funny with family,.... sometimes you want to say something, but you don't want to upset them by talking too much about it... So I stand back a little bit and bring it up when it's brought up, not too much. It's a very difficult thing. (9)

Some patients reported the effect that the physical effects of NMD had upon their sex lives.

> We've got to the point now where I can only really be in one position, that I **can't** get into by myself and I can't maintain for very long. But I mean, we do still try (*both laugh*). It's not as if it doesn't happen, but it's not necessarily very erm, very adventurous (3), or particularly exciting but, you know, it's, it's OK (*both laugh*). Erm, (4) but it can be frustrating because obviously I have to say, you know, "you're going to have to move my leg", or "get off that", you know, "you're hurting" (*laughs*) "move your arm, you're hurting me" (laughs). It's not very, erm... romantic on occasions. (14)

This influenced the self-image and emotional well-being of some patients.

> And our sex life, you know, it's a bit embarrassing really, but I used to love, making love to ----. You know, we had a good sex life.... and all of a sudden it's gone, you know. ----- says she doesn't mind, it doesn't bother her. I don't know whether it does or not. I can't tell really how she feels about it, but it makes me feel less of a man. I know that's silly, it's not the way to look at it, but it makes me feel, you know, like I'm not really... like I'm not really a proper husband. (15)

4.3.6 Impact of NMD on Social Participation and Integration

The visible physical impact and diagnostic label of muscle disease caused difficulties on a social level, which also influenced many patients' emotions and self-image.

4.3.6.1 Negative Social Reactions

Negative reactions from other people inhibited a number of patients in participating in activities of their choice.

> I did actually join a local gym, 'cos I felt like that was something that I could still do. But I just felt too out of place, I couldn't do the things I used to be able to do and there were a lot of posers in the gym and it was all glass fronted, so everyone walking past could see you and I just felt too self conscious ... (13)

4.3.6.2 Social Stigma

Negative social reactions reported were commonly due to other peoples' prejudices and misconceptions. For example, wheelchair users or those who were affected in their mobility or physical appearance were treated as if they were intellectually as well as physically impaired..

> You do get stared at to a certain extent, or people treat you as though you're an imbecile almost. They look at you and you almost feel as if they're going to pat you on the head. They do make you feel different yes, some do. (16)

> I was in the wheelchair at the hospital and the woman taking the appointments called over and asked a question... I answered and she said, "no, I am talking to your carer", and that really annoyed me because she was talking down to me and this is what hurts (2)

Patients in the CSP group reported this social stigma most commonly. One man attributed the negative reactions to other people's lack of understanding.

> they class you as..., they all put a name on everybody like, you, know, "weirdo" or whatever, you know. They don't understand, well...I didn't understand, still don't sometimes... (20)

Even patients with a congenital muscle condition who did not have obvious physical symptoms, reported stigma relating to the genetic nature of their condition. Some patients expressed guilt about having passed on their condition to their children, and patients reported sensing guilt in other family members. This further suggested the social stigma prevalent with regard to congenital muscle conditions.

> I almost sort of have this guilt thing, I was frightened of passing it on to **his** children, you know, poisoning his children with this horrible disease that I've got (17)

> My mother now has feelings of remorse that she's created these less than perfect children and she applies fault to herself..... (21)

4.3.6.3 Lack of knowledge & understanding

Other people's lack of understanding about disability and muscle conditions in particular were reported to cause problems in explaining their condition and distress in certain social situations.

> people don't like to talk about it, you know "don't mention it but she's got this muscle disease" and all this sort of thing. It's not a condition that people understand or know anything about. You can say "oh, I suffer from migraines" or "I suffer from hayfever" and everybody can understand ...but because it's a condition that is so rare... nobody understands it and, they don't know what you're talking about... (17)

Some patients suspected other people believed their ill-health to be feigned, which was also upsetting.

> One thing I do find is that people look at me, they think, "you're young, there's nothing wrong with you"..... A lot of people just assume that you're lazy, you don't work "Ah, there's nothing

wrong with you, what's wrong with you?" and that makes me feel **angry** that people are so shallow minded... (22)

This lack of understanding, also perceived in close family members, led to feelings of isolation as portrayed by one man with Myotonic Dystrophy.

There's no real support group I can contact, you know to find out... "Oh, you got the same as me, oh fine" .... if I've got somebody to talk to, it's nicer 'cos I can get it out, you know (*laughs*). This, just having somebody to talk to about it, I can't talk to the wife 'cos she doesn't really understand (8)

4.3.6.4 Stigma and discrimination

The fear of being stigmatised led some patients to cover up their condition.

There's no point in keeping it a secret 'cos they're going to find out eventually.... "Why can't this geezer shake hands? What's the matter with him?" "Oh sorry yeah, I get cramps". I don't tell them it's a muscle wasting disease, I don't say things like that, I just say "I've got cramp". (8)

Discrimination was reported, particularly with regard to employment and financial issues.

Some patients felt that their having a NMD influenced their job prospects.

I wait until I've proved I can do the job before I'll tell them. I don't usually tell people 'cos people just they think, "Ah, poor her" and I don't wanna be looked on as being different to everybody else 'cos in my view I'm not. People think, "oh God, she's got this" and I think it could affect things like promotion, so I just don't tell. If I feel that they need to know then I'll tell 'em, but most of the time I don't. (23)

4.3.6.5 Physical difference and associated social impact

The visible effects of NMD had a particular impact upon the self-image and confidence of a number of patients.

> I hate looking like this. I hate photographs of myself. I only ever look in the mirror if I absolutely have to. I don't like going out. I just hate the way I look, I hate being in this (*referring to wheelchair*). (14)

Negative body image was most evident in those who had undergone steroid treatment.

> I feel slightly embarrassed by being overweight. Just don't want to have people see me as getting overweight for the sheer hell of it . I feel quite strongly about that, 'cos I've always taken care of myself and I feel like I haven't had any control over that ..... It's like when you see somebody you haven't seen for a long time and you think, "gosh, haven't they changed", you know, and I hate the thought that people just might say "gosh, have you seen ------ lately? Hasn't she got fat" (1)

The impact of NMD and treatment side effects upon body image influenced the self-confidence and self-image especially in social situations. One lady diagnosed with Dermatomyositis reported the very specific symptom of skin rash and its influence upon her self-image.

> When I did go out when I was ill in the first place, I was extremely self-conscious about the rash and I knew I looked horrible, I just knew it. I hated myself and my self-confidence really went down... to me the rash was all there was of me, that's all I was, a walking rash. (24)

4.3.7 Expectations

Patients' expectations of the effects and consequences of muscle disease also influenced

their emotional well-being. Fears about losing independence and freedom were widespread in the interviews.

> It's very worrying 'cos up to now, I have steadily deteriorated, which means that in the future.... I probably will need even more help, which is very worrying... because, I know with the people I've visited in residential homes, their brain goes, they're in zombie-land. When you're in your own home you've got to do a certain amount of things for yourself, even if it's only to manage to go and make a cup of tea .... (11)

Many patients also made comments about the implications of a loss of independence for their partners or spouses.

> the only worry is in the future what my husband's got to cope with, looking after me. You know, if I deteriorate he might have to do more and more for me, that's my worry, but not myself personally..... (25)

4.3.8 Positive Experiences

Positive experiences were also reported and accounts given of how they had adapted to and learnt to accept their condition.

4.3.8.1 Relationships

Although dependence on other people was a common source of negative emotions, it also had a positive effect on some relationships. Some patients reported how their condition had brought them closer to other members of their family, particularly their partners or spouses.

I think it's brought us a lot closer really. I mean, my father brought me here today (*laughs*). They're always there to help me out and listen. I've sort of put them through a lot really and they stick by me..... My husband, it's probably put a lot of strain on our relationship, but I think in a way it's brought us closer as well ..... There's things that I want to do, around the house, maybe even something simple, like putting a picture up, that I can't do but I have to rely on him to do it, and he'll feel I'm nagging him because I'm asking him to do things all the time, but we find ways to work through it and I think in that way it's made us closer, 'cos we do talk about things more. (13)

4.3.8.2 Adaptation, Acceptance and Positive Outlook.

Some patients reported that their condition had enabled them to concentrate upon areas of their life in which they could achieve fulfilment. Many patients maintained a positive attitude and outlook.

I think it's made me more determined, in a lot of ways to actually get on. I'm more determined than my brothers and sisters.... they're not really bothered about jobs and careers and things like that really. It makes me want to be the best at everything that I can do. I'm quite a competitive person, but I'll only attempt things that I know I can do. (26)

as soon as we retired, I wanted to join dancing classes, well that's out of the window..... but there's lots of things that that's compensated by..... because I have to take extra rests then I can do my tapestry, I can do my oil paintings. So there's benefits that way. (27)

4.3.9 Disease 'impact' in congenital NMD patients.

Unlike patients with an acquired condition, some congenital NMD patients found it difficult to express how they had been 'affected' by their muscle condition in terms of any change in their physical functioning. They instead shared experiences of how they approached activities differently from other people and how they compensated for these

differences. One man reported how he lived with the condition without it having any perceptible impact upon his life.

> It's an odd condition... it's like it's a part of me... just like a leg or something and I don't miss being able to do the things a truly able bodied person is able to do... some of the functions I've lost I've managed to compensate for without even thinking (21)

To elucidate the experiences of muscle disease in patients in the CSP group, five supplementary interviews were conducted in these patients. From these it became clear that the influence of NMD on patients' lives were often experienced in terms of differences from the 'norm' or as a disadvantage compared to individuals not diagnosed with NMD. This was illustrated by the recollection of a young woman with Myotonic Dystrophy.

> When I was in school, teacher would say, I can't read your work.... write it again and again and again and I was like the only one and that sort of made me different from everybody else as well... (28)

The implications of having a muscle condition and its diagnostic label were relevant to the experiences of all NMD patients, but particularly those with a congenital condition. For some patients, being given a diagnosis provided an explanation for differences that the patient had always been aware of, for example in participating in sports at school.

> .... I sort of knew from an early age that there was something different about the way my body moved and I didn't know what it was. (18)

For example one lady with muscular dystrophy described the influence that being given a diagnosis had in providing a reason for a number of falls.

I- What's it like having an explanation for those things now?

P- Well I like it, I prefer to know why, rather than it just being that I'm clumsy, 'cos I used to think I must be stupid and clumsy for that to happen. (29)

4.4 Discussion

The analysis of the interviews revealed that the experience of muscle disease influences many areas of life. There were similarities across patients, but no set pattern of disease impact, particularly across the predefined subgroups of patients. Factors including age, gender, marital status, life circumstances, disposition and type of muscle disease undoubtedly influenced individual patients' experience and perception of NMD effects. The life domains that emerged correspond to the broad domains believed to make up the QoL spectrum (physical, psychological and social functioning) (Bowling, 1995; Group, 1995; Price, 1996).

The underlying determinants of the negative feelings expressed by patients included:

1. The **physical symptoms** of pain, fatigue, and muscle weakness and the effect of treatment upon these symptoms.
2. Perceived **degree of control** over physical functioning, social roles and emotions.
3. Ability to **participate** in activities and carry out plans of choice.

4. The effect of NMD upon **relationships.**
5. **Negative social reactions and stigmatisation** in response to the patient's physical appearance, their physical ability or to knowledge about the existence of their muscle condition.
6. Patients' **expected consequences** of their condition.

These issues have been documented in studies of patients with illness and disability. For example, physical symptoms of pain and fatigue have previously been found to contribute to lower levels of QoL (Albrecht and Devlieger, 1999). The effects of social discrimination and of an unaccommodating social and physical environment upon patients with disability are also well documented (Waddington, 1997) (Steinfeld and Danford, 1999). The stigma associated with physical differences and disability has also been discussed in depth (Goffman, 1963).

Patients with disability who report high levels of QoL have attributed this to having control over their bodies, minds and lives in general (Albrecht and Devlieger, 1999).

4.4.1 Relating findings to QoL Assessment

The life domains emerging from the interviews go beyond those captured by generic measures used in previous studies of muscle disease patients (Bach et al, 1991; Pehrsson et al, 1994; Ahlstrom and Gunnarsson, 1996; Ahlstrom and Sjoden, 1996; Ahlstrom et al, 1994; Drouet, 1996; Alexanderson et al, 1999; Chung et al, 2001) (See Table 4.5).

Table 4.5: List of domains emerging from in-depth interviews which have and which have not been incorporated in two generic QoL questionnaires

Domains incorporated in commonly used generic measures (NHP (Hunt et al, 1985), and SF-36 (Ware and Sherbourne, 1992)	Domains not incorporated in commonly used generic measures (NHP (Hunt et al, 1985), and SF-36 (Ware and Sherbourne, 1992)
Daily activities (ADL)	Physical symptoms (specific to muscle disease)
Social interaction	Relationships
Leisure activities	Perceptions of the future
Emotions	Employment
	Self-perception

This challenges the use of such measures, as any change in domains that are not included in these generic questionnaires would not be detected. Furthermore, certain items within generic questionnaires might not be relevant to the experience of NMD. For example items such as "Have you accomplished less than you would like?" (SF-36 Q5b) and "Have you been a happy person?" (SF-36 Q9h) are unlikely to be of specific relevance to NMD patients. These findings affirm the need to develop a new QoL measure, specific to patients with NMD.

Psychological adaptation and coping were exemplified in patients accounts of their efforts to maintain control and independence. Such processes are likely to influence patients' responses to QoL questionnaires along with factors such as disposition and life circumstances (Headey and Wearing, 1989; Brief et al, 1993; Feist et al, 1995; Allison, 1997; Albrecht and Devlinger, 1999). As patients reported varying degrees of acceptance and adaptation to their condition, a questionnaire developed for muscle disease should also take account of the dynamic nature of QoL.

The conceptualisation of QoL as 'the discrepancy between an individual's perceived current state and how s/he would like to be' (Calman, 1984; Cella and Tulsky, 1993; Group, 1995; Price, 1996) is consonant with idea of personal control and its influence upon patients' freedom, independence, self- image and emotional well-being. Patients with a high degree of control in their lives, regardless of physical disability, are likely to be able to fulfil many of their aspirations or conduct their lives the way they wish. This definition has already been used as the basis for a number of new questionnaires (Ruta et al, 1994; Group, 1995; Bradley et al, 1999) and provides a good foundation on which to develop questionnaires that will help to explain fluctuations in reported QoL.

It may be also be useful to delineate and separate the various stages of disease impact in QoL questionnaires. This could be done by questioning patients about the various stages of disease impact from symptoms and functional impact to effects upon self-image and emotions through to their overall evaluations of HRQL (Leventhal and Coleman, 1997; Hyland, 1992). Breaking down the process of HRQL evaluation (see section 1.8.2) should elucidate reasons for any change in HRQL and may indicate whether changes are due to a specific intervention or to adaptation.

4.4.2 Social Perspective

From the reported experiences of patients with NMD, it is clear that it is not only physical impairment and functional disability that cause difficulties in patients' lives. The patients interviewed, particularly those with congenital muscle conditions, did not always perceive muscle disease to have a particularly detrimental effect upon their lives. Instead they described how they approach certain activities differently to the able-bodied and avoid

some other activities altogether. In these cases the societal perspective is often more important in determining HRQL.

In light of the psychosocial impact evident in the patients interviewed, the revised ICIDH disability classification framework (ICIDH-2) promises to be of great value in enhancing the treatment and care of muscle disease patients. This framework acknowledges the contextual factors of the social and physical environment and also of patients' personal characteristics upon how disablement is experienced. The availability of social support (friends, family, patient support groups), technical aids and devices, high quality medical care and advice as well as accessible public facilities are just some of the factors that influence the degree of control and freedom that patients have in their lives.

By going beyond treatment at the level of physical symptoms and continuing to challenge misconceptions and prejudices and also social and environmental barriers, the impact of disease and disability may be significantly reduced. The more control and freedom patients have and the more opportunities there are for patients to integrate into society, the easier it will be to tackle the negative social attitudes that still isolate many people with illness and disability.

4.5 Conclusions

Exploration of patients' experience of NMD uncovered numerous effects that have previously never been recounted. These findings were helpful in designing the new QoL questionnaire. The life domains that emerged from the interviews influenced the content

of the questionnaire and the different ways in which disease impact was reported, from effects upon activities to influences upon patients' self image, supported its theoretical basis. This will be discussed further in chapter 8.

CHAPTER V

CONSTRUCTING QUESTIONNAIRES

Chapter 5: Constructing Questionnaires

5.1 Introduction

Following the exploration of patients' experiences in the qualitative interview study a postal survey was conducted to investigate the importance and prevalence of the life domains reported. Findings from the survey were used along with the interview results to develop the new QoL scale.

The project therefore involved constructing two questionnaires, a postal survey instrument and the final QoL questionnaire. It was therefore important to consider the issues involved in constructing the individual items and structure of questionnaires.

5.2 Composing questions

It is important that questionnaire items are easy to follow and understand. This can be achieved by making sure the questions are relatively brief, have straightforward phrasing and are free from jargon or ambiguity (Streiner and Norman, 1995; Mangione, 1995; Sudman and Bradburn, 1982).

5.2.1 Open-ended versus closed-ended questions

There are two main types of question that can be asked in questionnaires; closed and open-ended questions. Open-ended questions allow the respondent to provide his/her own answer whereas closed-ended questions require respondents to select a response from predetermined categories.

Standardised questionnaires customarily adopt closed-ended questions, whereas individualised scales tend to use more open-ended questions that allow respondents to voice individual concerns.

5.2.1.1 Open-ended questions

Open-ended questions can be useful in exploring a topic when developing new questionnaires (Sudman and Bradburn, 1982). However, they involve greater effort to complete and may be intimidating to respondents with lower levels of education or literacy. It has therefore been suggested that open-ended questions be included only as an appendage to closed-ended questions (Mangione, 1995). However, the provision of space for comments may be an incentive to respond as it allows respondents to voice their opinions without being constrained by predefined response options (Moser and Kalton, 1971).

5.2.1.2 Closed-ended questions

Closed-ended questions require people to respond within predetermined categories and therefore risk forcing answers into inappropriate categories. Despite this, their completion involves less effort than for open-ended questions, which means they are more likely to be completed. They are also easier to analyse as scoring schemes for closed-ended questions are predetermined.

5.3 Scaling Response Categories

5.3.1 Binary Scales

Binary scales, (e.g. scales that offer response options of 'yes' or 'no') such as the Quality of Well-Being (QWB) Scale (Kaplan et al., 1976) and the Sickness Impact Profile (SIP) (Bergner et al., 1981) are simple but do not represent intermediate points

between the extremes of a continuum (e.g. depression). This means that they are less sensitive to change and they are less acceptable to respondents given that the effects of illness upon QoL are unlikely to be experienced in an all or nothing way.

5.3.2 Categorical/Likert Scales

Likert scales (Likert, 1952) ask respondents to indicate their agreement or disagreement with a statement on a scale consisting of adjectival categories that range from, for example; 'strongly agree' to 'strongly disagree' or 'not at all' to 'extremely' (Figure 5.1).

Figure 5.1

Please circle one of the boxes from the scale below

STRONGLY AGREE	AGREE	NO OPINION	DISAGREE	STRONGLY DISAGREE

These scales are used in questionnaires such as the SF-36 (Ware and Sherbourne, 1992; Brazier et al., 1992) and have the advantage of being simple and almost universally understood.

Unfortunately, Likert scales are labelled with adjectives that may be interpreted differently by different respondents. This may addressed by numbering the scale in order to make the intended order of categories more intuitive (Figure 5.2).

Figure 5.2

STRONGLY AGREE	AGREE	NO OPINION	DISAGREE	STRONGLY DISAGREE
1	2	3	4	5

However, numbering categories may add additional bias and may influence responses depending upon the numbering applied to the scale. For example, scaling the item from –5 to +5 has been found to result in fewer respondents using the lower half of the scale compared to a scale ranging from 0 to 10 (Schwarz et al., 1991). Care should therefore be taken to ensure that scale numbering does not result in skewed data.

5.3.3 Visual Analogue Scales

Visual analogue scales (VASs) are lines of a fixed length (usually 100mm), anchored at either end by the extremes of the scale (e.g. 'none at all' & 'extreme'). Responding involves marking a line or a cross on the scale at the point that represents their position between the anchor points (Figure 5.3).

Figure 5.3

Please mark a cross on the scale below to indicate how bad your pain is.

VASs do not confine responses to previously defined categories and as such were adopted to achieve greater accuracy and sensitivity to change than categorical scales. VASs have become popular over the last couple of decades in rating symptoms such as pain (Huskisson, 1982) and they are also used to measure other subjective states, including depression (Lees and Lloyd-Williams, 1999) and fatigue (Brunier and Graydon, 1996).

5.3.4 Graphic Rating Scales

Graphic rating scales (GRSs) are similar to VASs except that they are graded with numbers or adjectival labels at intervals along the line (Figure 5.4).

Figure 5.4

Please circle the number that best describes your degree of pain on the scale below.

NO PAIN	0	1	2	3	4	5	6	7	8	9	10	EXTREME PAIN

These scales are easier to complete than the VAS, involving circling a number or descriptive label. They are also easier to score as, rather than measuring the distance between the line or 'X' and the anchor point at the low end of the scale, scoring simply involves taking note of the number selected.

Numerical rating scales (e.g. Figure 5.4) have been found to be more responsive to change in pain than VASs or verbal rating scales (Bolton and Wilkinson, 1998) which suggests their usefulness in evaluative studies such as treatment trials.

5.3.5 Visual Analogue versus Categorical Scales

A number of studies have found little difference in the efficacy of visual analogue and categorical scales (Remington et al., 1979; Slevin et al., 1988). However, the lack of familiarity with the VAS has resulted in more inaccuracies in completing these scales. One study found that 7% of respondents had completed VASs inaccurately, compared to 3% for graphic rating scales (Huskisson, 1974). It has therefore been recommended that respondents be taught how to complete these scales. However, this is more time consuming and means that VASs are inappropriate for use in postal survey instruments (Guyatt et al., 1987).

Bias has also been reported in the completion of VASs. Factors such as the orientation of the scale (either horizontal or vertical) and labelling (e.g. with lines or verbal descriptors) have been found to influence the distribution of responses on these scales (Paul-Dauphin et al., 1999), casting doubt on the validity of this scaling method.

Another disadvantage is that changes on VASs are difficult to interpret clinically. For example, the meaning of a change of 10mm or 20mm on a VAS is not clear, whereas changes on a Likert scale demonstrate an interpretable shift from one category to another (Guyatt et al., 1987).

Given the problems with VASs and the greater familiarity and ease with which categorical scales can be completed and scored (McQuay, 1990), categorical scales are often a more attractive choice for clinicians and researchers.

5.3.6 Is there an optimal number of response options?

It is beneficial to adopt a scale with more than two response categories (e.g. 'yes/no'). Binary scales can be frustrating for respondents and as they often do not represent the full spread of variation between individuals. Reliability has been found to decrease the fewer the categories are used in the scale, although the loss in reliability was found to be small in scales of more than 7 categories (Nishisato and Torii, 1970).

People also have difficulty discriminating between more than seven categories (Miller, 1956), although the tendency for respondents to avoid extremes on the scale ('end aversion bias') has resulted in recommendations of a 9 level scale (Streiner and

Norman, 1995). This is less appropriate for Likert-type scales in which adjectival descriptors for each level would result in a cumbersome scale. Even reducing the number of levels to five or even three is unlikely to result in a significant loss of information when there are a large number of items on the scale (Streiner and Norman, 1995). Between 5 and 7 categories are therefore recommended as the optimal number of response options (Streiner and Norman, 1995; Fowler, 1995).

In categorical Likert scales, odd and even numbers of categories have been shown to have similar efficacy (Remington et al., 1979) and are suited to different purposes. Scales that provide an odd number of categories allow respondents to provide an answer in the middle of the scale (i.e. indicating "no change", or "neither good nor bad"). However, the use of an even number of categories to force choices one way or the other may be appropriate in the likes of opinion polls or attitude surveys.

5.4 Timeframe

Some measures do not define the timeframe (period of time referred to in questionnaire items). Others may ask the respondent to consider the previous day, week, or month with regard to his/her response. Specifying the timeframe ensures that respondents refer to a particular point of time in order to give a representative picture of the situation since that time. This means that the information gathered will be more valid and changes over particular periods of time are more likely to be detected. It has been suggested that timeframes of a few days are preferable to those that ask about the past few weeks as they are less subject to inaccurate recall (Osoba, 1998).

5.5 Questionnaire Length

Shorter questionnaires are believed to yield better response rates than longer instruments (Mangione, 1995). Brevity is a particularly important attribute in health status scales as it is estimated that patients in ill health become tired after about 15-20 minutes (Osoba, 1998). On the other hand, longer questionnaires may be seen as more important or interesting (Mangione, 1995) and may motivate more people to respond.

Despite this, the influence of questionnaire length is likely to be negligible once a certain number of pages have been reached. For example, there was no difference in response to a 28 page questionnaire, compared to a 32 page questionnaire that contained 59 extra questions and took an extra 5-10 minutes to complete (Kolowski, 2001). It is believed that as long as the subject of the questionnaire is salient and respondents have a reasonable level of education it is possible to maintain response levels to questionnaires of 12-16 pages (Sudman and Bradburn, 1982).

5.6 Format of the questionnaire

Questionnaires should have a short and simple introduction, questionnaire items should be easy to read and uncluttered and completion of the scale should be easy, with tick boxes or numbers to circle. A booklet format looks more professional and should minimise loss of information through respondents missing pages (Sudman and Bradburn, 1982). Finally, a thank you statement should be provided at the end of the questionnaire.

5.6.1 Branching questions or 'skip' patterns

In most questionnaires or surveys individuals may be required to skip over questions that do not apply to them (e.g. unmarried individuals to skip the questions on relationship with spouse). The problem with this is that branching questions can be confusing and may result in missed items (Fink and Kosekoff, 1998). It is therefore important for instructions to be clearly presented, with the use of arrows, boxes or bold font. Instructions to skip to a question on the same page or the top of the next page are also helpful. Such measures should save respondents the time and effort required in attending to irrelevant questions.

5.6.2 Ordering of questions

Questions should be placed in a logical order and grouped together with transitions provided to describe the upcoming set of questions (Fink and Kosekoff, 1998). In order to minimise item non-response, non-threatening, salient questions should be placed at the start (Sudman and Bradburn, 1982) as respondents may be put off by more challenging questions. However, sensitive or difficult questions should appear well before the end, as respondents may become tired particularly if the questionnaire is long and difficult. Demographic questions (e.g. gender, age) should be placed at the end (Sudman and Bradburn, 1982). These tend to be easy to answer but may also be threatening or off-putting to some respondents. Placing them at the end means that they will be completed without influencing responses to the other items.

5.7 Conclusion

The principles outlined here were put into practice in constructing both the postal survey instrument (chapter 7) and the new QoL questionnaire (Chapter 8).

CHAPTER VI

CONDUCTING POSTAL SURVEYS

Chapter 6: Conducting postal surveys

6.1 Why conduct a postal survey?

Postal surveys are an efficient and cost-effective way of investigating an issue in a large number of people within a short space of time. They require minimal staff and facilities and are particularly useful in gathering information from rare populations and widely dispersed samples (Fowler, 1995).

The survey method was particularly useful in this study as surveys can help to verify the analysis of qualitative studies in a larger, more representative sample. If surveys also incorporate open-ended questions, they can provide supplementary qualitative data about the opinions or experiences that may not have emerged during qualitative investigation.

Surveys also have the advantage of giving respondents time to provide thoughtful answers (Mangione, 1995; Moser and Kalton, 1971). They also avoid the biases introduced with the use of an interviewer as respondents are more likely to answer questions of a personal or embarrassing nature and give less socially acceptable responses (Moser and Kalton, 1971).

6.2 Pilot testing

Reading through questionnaire items and trying to hear them from a naïve perspective can be useful in detecting any confusing questions or instructions, or issues that may have been left out. Feedback from colleagues prior to the pilot study may also be helpful.

Pilot testing is essential in the development of postal survey questionnaires as it allows the investigator time to reorganise any problematic parts of the instrument (Litwin, 1995) and determine whether the survey will provide the information required (Fink and Kosekoff, 1998).

Pilot studies allow the detection of any typographical errors in the questionnaire, problems in layout or ambiguity in the instructions or questions (Litwin, 1995; Fink and Kosekoff, 1998). They are also useful for ensuring that the vocabulary used and content of the questionnaire is appropriate, the format flows well and that the questionnaire is of a reasonable length (Litwin, 1995).

Pre-testing should also minimise the use of awkward or embarrassing questions, or an unhelpful layout. This should help enhance response rates and avoid biased responses (e.g. social desirability bias) (Fowler 1995).

6.3 Conducting the Survey

6.3.1 Timescale

It is recommended that one month should pass between the original mail out of the survey to the analysis of the data (Moser and Kalton, 1971). A large number of responses will be received within two weeks but as many will be returned late, time should be allowed for responses to follow-up attempts.

6.3.2 Sampling

The sampling method adopted in postal surveys depends largely upon the population of interest. If the general population is being surveyed, it may be more appropriate to use a stratified sampling method (Coolican, 1994) so that the general population can be adequately represented. This involves delineating all the subgroups within the

population for demographics such as gender, age, race and socio-economic status and attempting to represent each of these in the sample selected.

In this study a convenience sampling method was adopted which involved drawing the sample from a group that was ready and available. This was appropriate given the relatively small number of NMD patients and consequent need to sample patients from a large geographical area.

6.3.4 Confidentiality

Coding for identity is helpful in enhancing confidentiality (Fink and Kosecoff, 1998). Forms or return envelopes can be marked with an identification number rather than the respondent's name. These numbers can then be linked with the list of names to whom the survey was sent and reminders can be sent to non-responders.

6.4 Difficulties in Postal Surveys

6.4.1 Item non-response

Item non-response occurs when respondents do not know the answers to particular questions or when they refuse to answer confusing, embarrassing or irrelevant questions (Fowler, 1995). Respondents may also miss questions out or provide their own answers if presented with too few response categories or if the categories do not represent their opinion or position. This is problematic if a large number of respondents leave certain questions unanswered. Rigorous pilot testing should ensure the acceptability and lucidity of the questionnaire and help to minimise non-response.

Other methods used to address the issue of item non-response include imputing scores for missing items based on responses to other items on the scale. This can be useful if

a few items are left incomplete but is less appropriate if a large proportion of responses are missed (see Fowler, 1995).

6.4.2 Low response rates

Compared to interview studies, postal surveys do not have the immediacy and incentive to ensure a good response rate, making non-response a common setback in postal survey studies.

There have been postal surveys reported in which only 5% to 20% of the sample responded. These are unlikely to give a fair representation of the population under study (Fowler, 1995). Researchers tend to aim for a response rate of 70% or more (Fink and Kosecoff, 1998) in order to achieve a representative picture of the issue under study.

6.4.3 Non-responders

People not responding to surveys include:

- Those who did not receive the questionnaire, for example those who have changed address.
- Those unable to complete or respond to the survey due to ill health, inadequate reading or writing skills, or skills in the language of the questionnaire.
- Those who did not wish to complete and return the survey
- Those who did not get around to completing or sending off the survey

It is therefore important to check the list of respondents to ensure that it is up to date. Unfortunately, little can be done to address non-response from the illiterate or those physically unable to respond. By conducting pilot studies to ensure that the

questionnaire is straightforward and easy-to-complete more people will be able to respond.

Making the survey more attractive and interesting, and emphasising its importance also encourages respondents to reply. Finally, a rigorous follow-up of the initial mail shot with reminders and repeat mailings should help to minimise non-response. This should be particularly helpful in maximising response from those who fully intended to reply but may not have got around to doing so.

6.4.4 Bias due to Non-response

Postal surveys exclude certain respondents and may lead to bias in the results and the conclusions drawn from them.

Response may be influenced by:

- The respondents' ability to complete the scale
- The area in which the individual or sample lives. For example, response rates tend to be higher in rural areas than in cities.
- Income level and social class.
- Level of education. For example, those who are less educated may be less able to complete certain scales or may feel intimidated by the survey process.
- Age. For example, the elderly may have more trouble in completing the form.
- Gender. It is has been suggested that males may be less co-operative as a group than females.
- Level of motivation and degree of interest in the research.

(Fowler, 1995; Moser and Kalton, 1971).

Therefore, if the survey is interesting and of relevance to the population of interest there is likely to be a better response. The researcher has less influence over demographic factors such as age, gender and social class. However, a rigorous follow-up should encourage response from the subgroups that tend to be underrepresented.

The limited control that researchers have over surveys means that there is no way of telling whether someone other than the intended recipient completed the questionnaire. This is another important source of bias in survey research.

6.5 Achieving good response rates

The main difficulty in postal surveys is inducing respondents to complete the questionnaire without the intervention of an interviewer. A 20% response rate for the first mailing is not uncommon. However, it is suggested that this can be elevated to 70 or even 80% through repeat mailings and reminders (Fowler, 1995).

6.5.1 Appearance of the Questionnaire

Measures that can be taken to improve response rate include making the questionnaire look more attractive (e.g. using a visually attractive layout, coloured paper) and professional (e.g. printing the questionnaires professionally) (Fowler, 1995). This may make respondents see the project as more important and worthy of their time.

Including a good covering letter and a stamped addressed or business reply envelope along with the questionnaire should also help. The letter should convey the importance and usefulness of the research, explaining the study as well as motivating respondents to take part. Emphasis should be placed on confidentiality and that

participation is voluntary (Mangione, 1995). The covering letter should also be brief (one page), and typed on the headed notepaper of the supporting institution. Providing a contact name and telephone number ensures that respondents can contact the researcher if they need assistance in completing the form and signing the letter in blue ink makes it seem more personal and may increase incentive to respond.

6.5.2 Incentives

Prepayment to individuals has been found to enhance response rate but may not be appropriate for certain studies. Monetary or other material incentives are better reserved for more consumer-oriented, or market research-style surveys rather than those enquiring about peoples' experience of disease.

6.5.3 Ensuring confidentiality of responses

Measures to ensure the confidentiality of participants' answers are also believed to enhance response rate (Mangione, 1995). However, complete anonymity may be impractical as it means that identification numbers have to be left off the forms and the survey resent to those who have already responded, as well as non-responders.

6.5.4 Repeated contact

Repeated contact with respondents is the most important factor in enhancing response rate (Fowler, 1995). In order to achieve a 75% response rate it is recommended that at least four mailings are performed (Fowler, 1995) enclosing the complete package (questionnaire etc) in the first and third mailings (Fink and Kosecoff, 1998) and a postcard or letter reminder in the second and fourth (Mangione, 1995).

Contacting non-responders by telephone may also encourage response (Kolowski et al., 2001). However, this may be costly and difficult to implement if potential respondents can not be contacted or if telephone numbers are unavailable.

6.6 Addressing low response rates

If a low response rate is achieved despite measures to maximise response, proxy respondents can also be surveyed. Unfortunately, this is not as good as self-report particularly for topics involving subjective information such as feelings, knowledge or opinions (Fowler, 1995).

In order to gauge whether the data is representative, non-responders can be surveyed and their characteristics compared to those who respond to the first round of data collection (Fowler, 1995). Data gleaned in repeat mailings can also be used to estimate the direction and amount of bias in the data from the first phase of data collection. If the new round of data collection replicates the initial phase, the researcher can be more confident that the data are representative.

6.7 Conclusions

It is clear from the literature that careful planning and a rigorous approach to the mailing and follow up of questionnaires is essential to the success of a postal survey study.

Having explored the experiences of individuals with NMD it was important to verify these findings in a larger group of individuals and glean any additional information for use in the new QoL questionnaire.

The postal survey was conducted following the recommendations outlined in this chapter and is described in chapter 7.

CHAPTER VII

EXPLORATION OF PATIENTS' PERCEPTIONS OF NEUROMUSCULAR DISEASE IMPACT: A POSTAL SURVEY INVESTIGATION

Chapter 7: Exploration of Patients' Perceptions of Neuromuscular Disease Impact: A Postal Survey Investigation.

7.1 Introduction

It has already been established that QoL questionnaires should be constructed on the basis of both theory and exploratory research (Chapter 2). The results of the initial exploratory interview study engendered a better understanding of patients' experiences and provided a basis upon which to conduct more quantitative exploration. This survey aimed to confirm and elaborate upon the findings of the interview study and to uncover any issues that may have been neglected. It was felt that investigating the effects of NMD in a larger sample would provide a more representative picture of disease impact. In order to achieve this the prevalence of impact across life areas and the importance attributed to this were investigated. This also facilitated the selection of the most relevant items for the final QoL questionnaire, ensuring as accurate and responsive a scale as possible.

Respondents were also asked whether the effect of their NMD upon each aspect of life was positive or negative. This method was adopted as positive effects of illness are commonly overlooked in QoL assessment despite findings of high levels of QoL in cancer patients (Watson and Pennebaker, 1989; Fromm al, 1996; Taylor et al, 1984) and patients with disability (Albrecht and Devlieger, 1999). Symptoms such as pain have been also been found to have a positive effect upon family relationships (Padilla, et al, 1990), therefore it was considered important to include this method.

7.2 Methods

7.2.1 Devising the Postal Questionnaire

The postal questionnaire (Appendix B) was designed around the life domains extracted from the interviews. The survey questions probed the following domains.

1. Daily activities
2. Employment / working life
3. Social & Leisure activities
4. Relationships with friends
5. Family relationships
6. Relationship with spouse/partner
7. Relationships with other people (strangers, acquaintances and colleagues)
8. Independence
9. Emotions
10. Body image
11. Perceptions of the future
12. Treatment

These questions relate to the domains listed in Chapter 4 (p. 91, table 4.4). The only difference is that, for the purpose of the survey, the relationships domain was split into the sub-domains of friends, partner /spouse and family.

Questions about treatment were also included, facilitating the investigation of beneficial effects as well as the side effects of treatment, issues that were touched upon in the interviews.

7.2.2 Format of the Questionnaire

The survey posed a series of closed ended questions to determine the extent to which people are affected by their condition and the importance they attach to disease impact across various life domains.

Boxes for comments were also incorporated to allow respondents to share their individual concerns and experiences. This was done to ensure comprehensive coverage and the detection of any additional issues that may not have emerged in the interview study.

7.2.2.1 Impact of NMD

The degree of impact in each area was measured by responses provided on a five point Likert scale. Response options ranged from 'not at all' affected to 'very much' affected. Five point scales were believed to be suitable for registering whether patients experience an impact, the degree of the impact and its perceived importance. Any additional scale categories would have been unnecessary for this type of survey instrument.

7.2.2.2 Positive or Negative effect

The nature of NMD impact was probed further with respondents asked to indicate whether the overall effects of their condition were positive or negative.

7.2.2.3 Importance of Impact

Questions about the importance of disease impact were incorporated, one for each life area. Again, responses were scaled along a five point Likert scale, ranging from 'not

at all important' to 'extremely important'. This was done to give an idea of the salience of the issues reported by patients, an important consideration when selecting items for inclusion into the questionnaire.

7.2.3 Pilot Study of the Postal Questionnaire

The postal instrument was piloted to test the acceptability of the scale and ensure patients' comprehension of question wording, layout and rating scheme.

7.2.3.1 Methods

The questionnaire was piloted in 11 patients attending a routine follow up muscle disease clinic or for a separate research appointment.

Patients were questioned about the clarity of the instructions, the length of time taken to complete the scale, and the appropriateness of the questions (Litwin, 1995). They were also asked whether there were issues that they would like to have been asked about that were not included in the questionnaire and if there were any questions that they felt were irrelevant. Changes that were made following completion by the first five patients were piloted in the remaining six respondents.

7.2.3.2 Results

Respondents found the survey instrument to be easy to complete, although a few did not easily understand questions about the positive or negative effects of NMD. Some patients also found it difficult to classify the effects of their condition as simply either 'positive' or 'negative'.

The treatment section caused no problems although two patients expressed a desire to comment upon why they did not receive treatment

7.2.3.3 Adaptations to the Postal Survey Instrument

Minor changes were made to the survey instrument as a result of the pilot study (Appendix C). To address difficulties with questions about the 'positive' or 'negative' effects of NMD, these words were substituted with the words 'good' and 'bad'. Furthermore, in order to address difficulties in expressing the effects of NMD as either good or bad, an additional option was included, allowing patients to tick an 'other' category and then to express the impact of their condition in their own terms.

The treatment section also changed to include a question to allow those not in receipt of treatment to express why they did not receive treatment and how they felt about this.

These changes were found to be acceptable to the remaining six patients taking part in the pilot study.

7.2.4 Postal Survey

The postal questionnaire was sent out to 537 patients with NMD. 480 of these were sent through patient support groups in order to reach a large number of patients. This also maintained patient confidentiality. The remaining 57 were sent to patients from King's College Hospital (KCH) clinics.

380 questionnaires, coded to ensure the respondents' anonymity, were sent through the Myositis Support Group (MSG) to their adult members (all those age 18 years and

over). The MSG estimated this membership to comprise 200 Dermatomyositis patients, 150 Polymyositis patients (ARR subgroup of patients), and 30 or more patients with Inclusion Body Myositis (ASP subgroup).

100 questionnaires were sent to patients with facio-scapulo-humeral muscular dystrophy through the FSH-MD support group and a further 57 questionnaires to patients with a variety of congenital NMDs, from King's College Hospital (KCH) clinics.

A second mail out of the questionnaire took place three weeks after the first. The identification codes marked on the questionnaires ensured that the second questionnaire was sent only to those patients not responding within the three-week period. Both mailings included prepaid envelopes in which the respondents could return the questionnaire.

7.2.5 Analysis

7.2.5.1 Quantitative Analysis

The quantitative data was analysed using the Statistics Package for Social Scientists (SPSS). Descriptive statistics were used to calculate the prevalence and importance of NMD impact to patients across the life domains covered in the survey.

7.2.5.2 Qualitative Analysis: Comments about specific life areas

Thematic analysis was conducted upon the comments written in the survey questionnaires to corroborate and verify the qualitative interview data. The analysis followed the same principles as the analysis of the interview transcripts (see chapter

4). Themes were drawn out and clustered into broader categories representing different reported effects of disease and impact upon specific life domains.

7.3 Results

7.3.1 Sample

252 completed questionnaires were returned in total (response rate 47%). There was a 50 % (n= 78) response rate from the 157 patients with a CSP NMD. 42% (n=146) responded from the estimated 350 patients with an ARR disease, and approximately 93% (n=28) responded from the estimated 30 or so patients with an ASP NMD.

The male to female ratio of the sample was: - 1: 2.8

Table 7.1: Age of the survey sample and duration of NMD

Group (Number disclosing date of birth. 4 respondents did not disclose this information)	**Mean age (in years)**	**Standard deviation**	**Age range (in years)**	**Duration of condition** - *estimated from reported time of diagnosis* **(Mean in years)**
All respondents (N=248)	52.6	16	16-96	11.8
Male (N= 65)	55.9	16.3	22-82	14.1
Female (N=183)	51.4	15.8	16-96	11.1
CSP (N=76)	44.4	15	16-82	19.8
ARR (N=145)	53.6	14.4	22-82	9.5
ASP (N=27)	70.5	10.2	44-96	7

7.3.2 Positive and Negative Impact

All the items in the survey included a question that probed to see whether the impact upon each life area was positive, negative or whether it had an 'other' kind of effect. The proportion of respondents rating the impact upon any one domain as positive was very low. For all the domains except the relationship and social interaction items, only 2 to 4% of respondents reported a positive effect of NMD. For the social

interaction and relationship domains between 5% (for impact upon partner/spouse relationship) and 11% (for Friends item) and patients rated the impact of NMD upon these as 'good'. A few more respondents selected the 'other' option but almost exclusively described the effect of NMD as being in some way negative (e.g. 'devastating', 'difficult') or as 'mixed'.

7.3.3 Life Domain Items

Over 60% of all respondents reported an impact upon the life areas included in the questionnaire (Figure 7.1). Indeed, in all the domains except family, friends and social interaction, over 75% of the respondents reported being at least 'slightly affected'. The highest impact ratings of 'quite a lot' and 'very much' were selected by a large number of respondents across all the domains (Figure 7.2), especially the activity domains and the psychological impact domains of independence and emotions. The relationship and social interaction items received fewer ratings in the highest impact categories, although ratings were still high. Around 30-40% of respondents selected these categories.

Over 90% of all those who reported an impact also reported this impact to be important (Figure 7.3). The chart showing the number of respondents selecting the highest importance ratings of 'very' or 'extremely important' (Figure 7.4) is somewhat more telling. Independence received the highest importance ratings following by Perception of the Future and Working Life. Social Interaction received the fewest ratings of 'very' or 'extremely important', with 50% of respondents selecting these ratings.

Figure 7.1: Patients reporting an impact upon each of the life areas

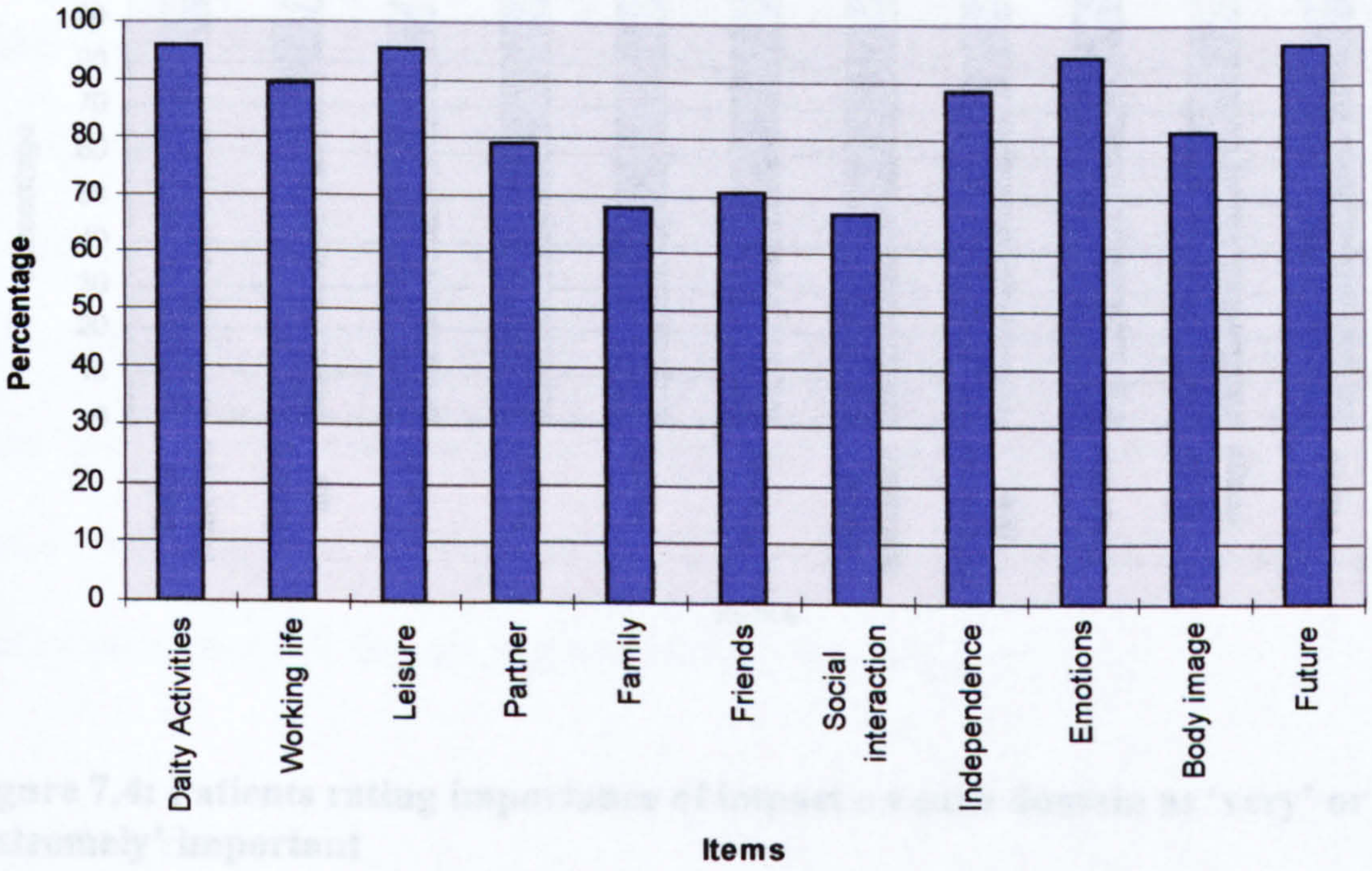


Figure 7.2 Patients reporting impact on life domains as 'quite a lot' or 'very much'

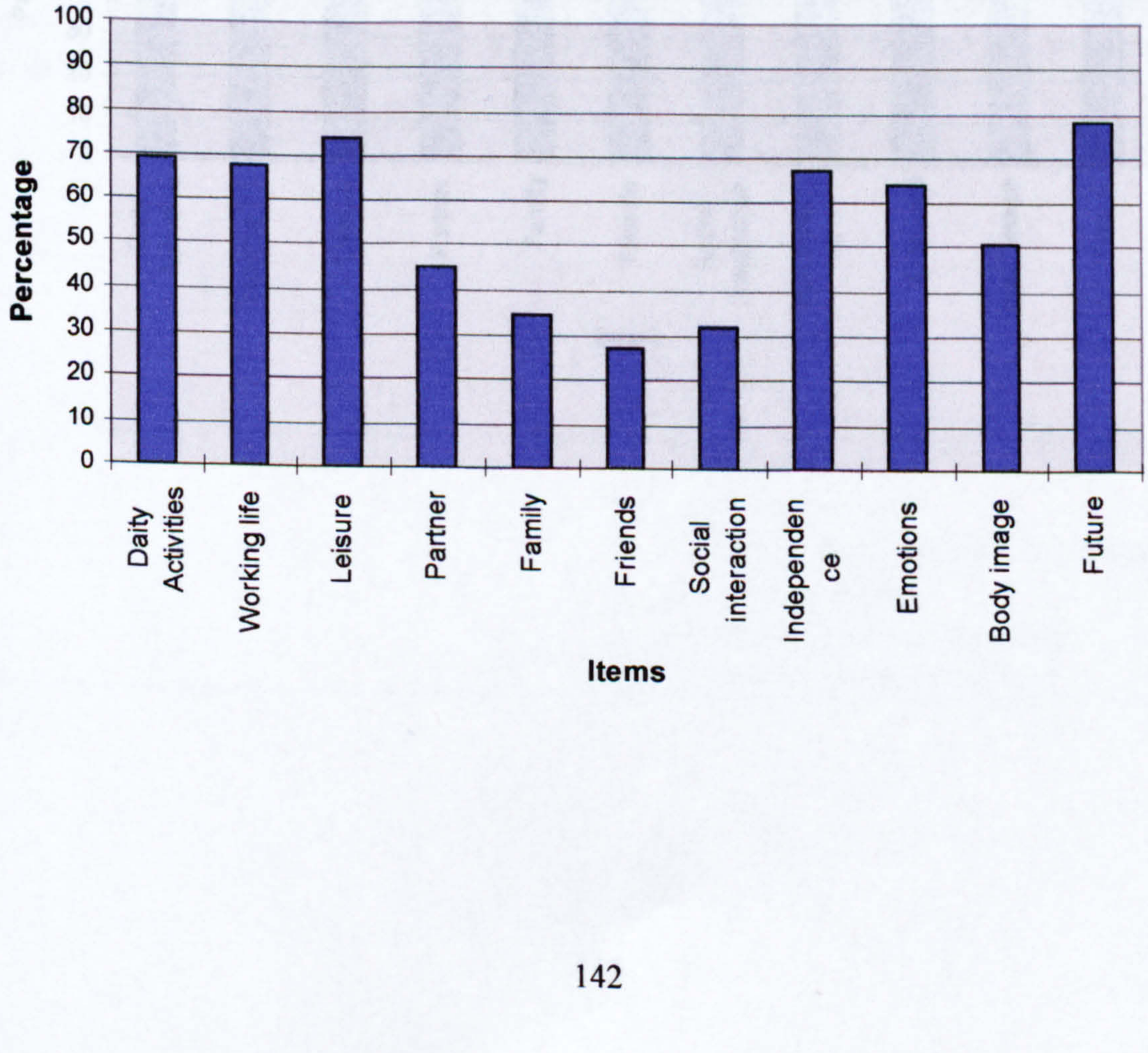


Figure 7.3: Patients rating impact on each domain as important

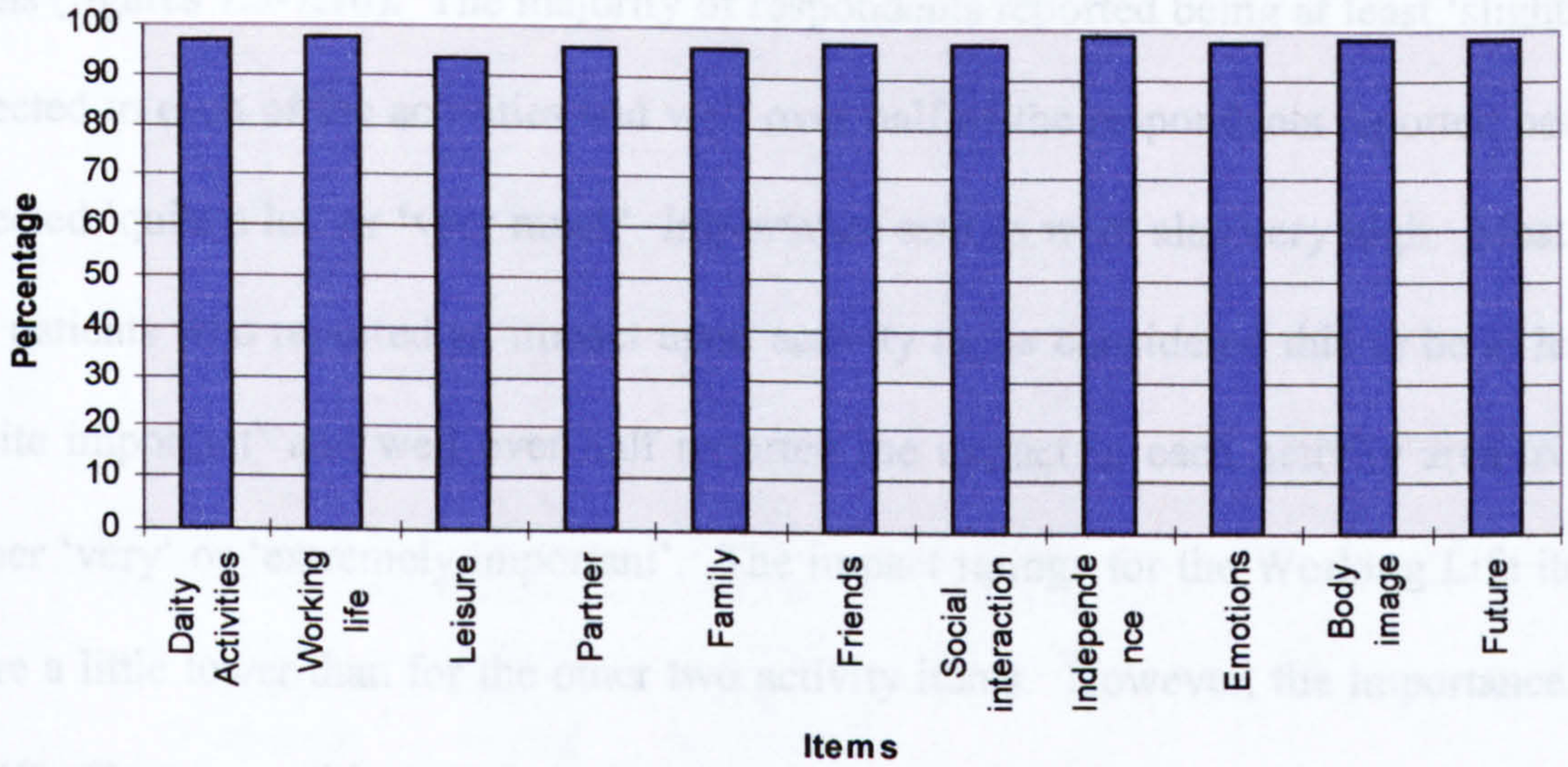
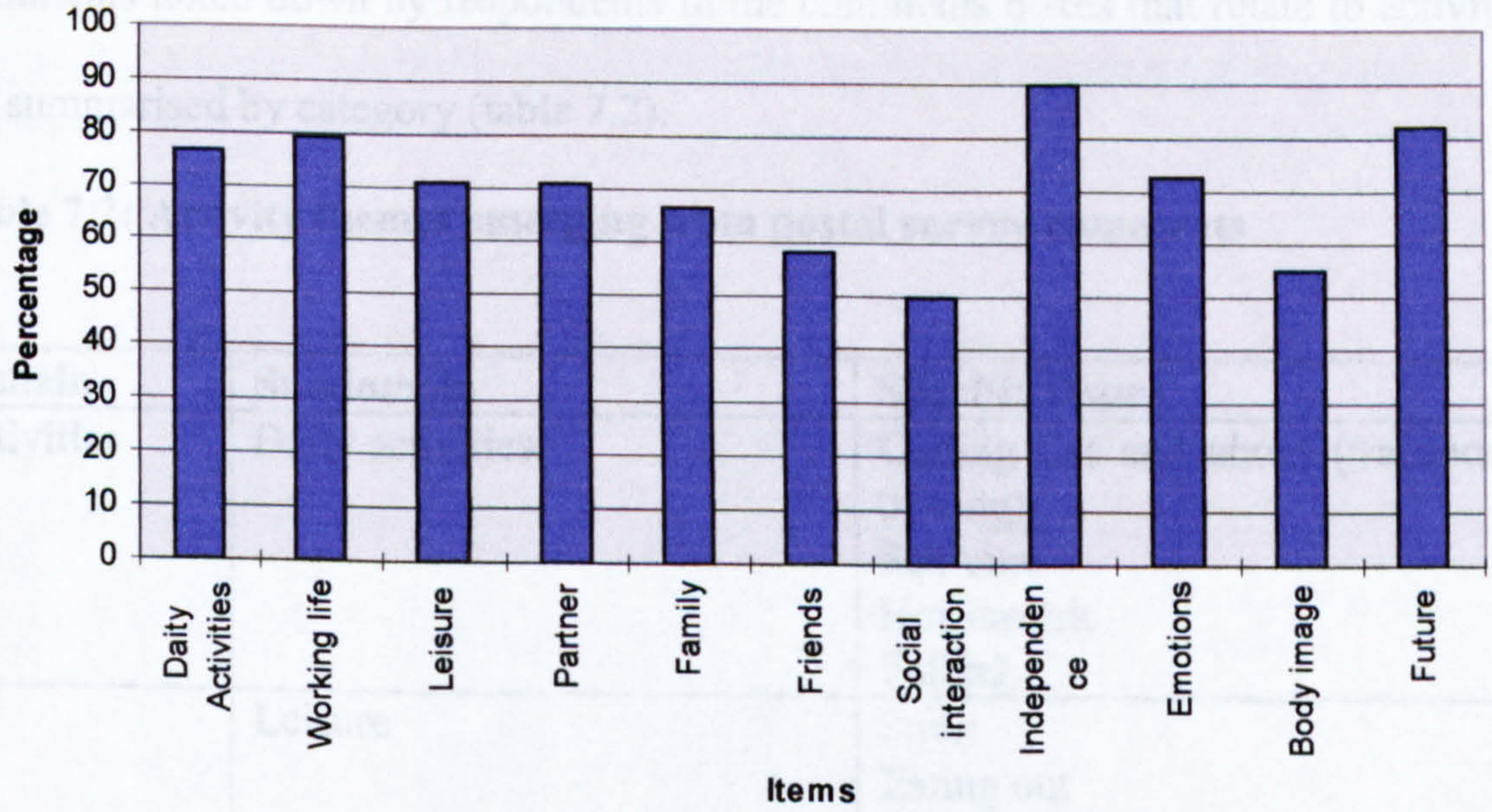


Figure 7.4: Patients rating importance of impact on each domain as 'very' or 'extremely' important



7.3.4 Activities (questions 1-3)

The percentage of respondents reporting an impact was high across all the activity items (figures 7.5-7.10). The majority of respondents reported being at least 'slightly' affected in each of the activities and well over half of the respondents reported being affected 'quite a lot' or 'very much'. Importance ratings were also very high. Most of the patients who reported an impact upon activity items considered this to be at least 'quite important' and well over half reported the impact in each activity area to be either 'very' or 'extremely important'. The impact ratings for the Working Life item were a little lower than for the other two activity items. However, the importance of NMD effects upon this domain in those working was very high.

Qualitative Data

Comments noted down by respondents in the comments boxes that relate to activities are summarised by category (table 7.2).

Table 7.2: Activity themes emerging from postal survey comments

Domain	Subdomain	Specific Issues
Activities	Daily activities	Getting out and about (transport & driving) Self care Housework Falling
	Leisure	Sport Eating out Sedentary hobbies Visiting friends & family
	Employment	Work activities Stopping work Limitations to career/ job prospects (Discrimination) Change in job/ occupation Financial considerations

Figure 7.5: Impact on daily activities as reported by survey respondents

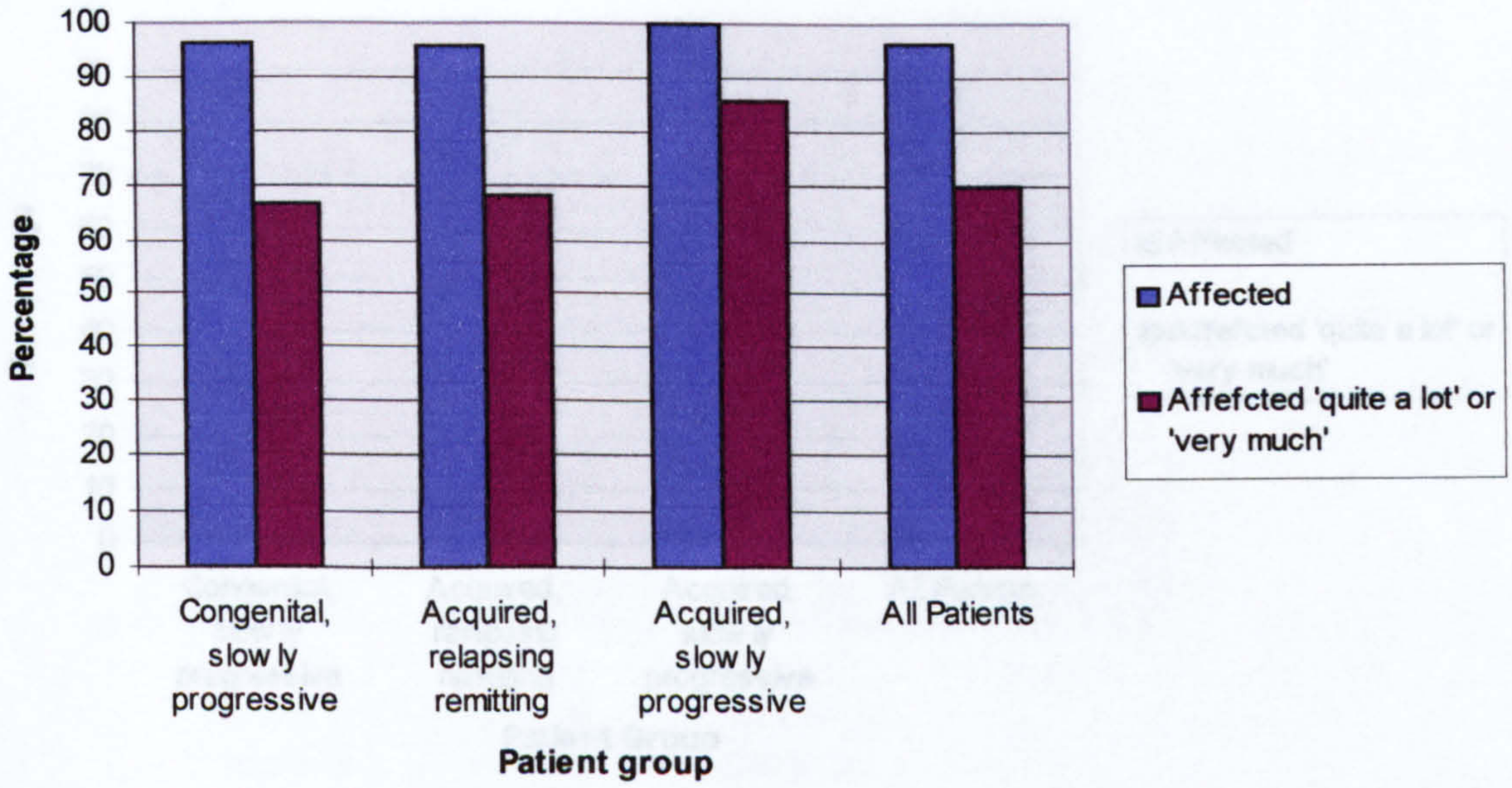


Figure 7.6 Importance of impact upon daily activities as reported by survey respondents

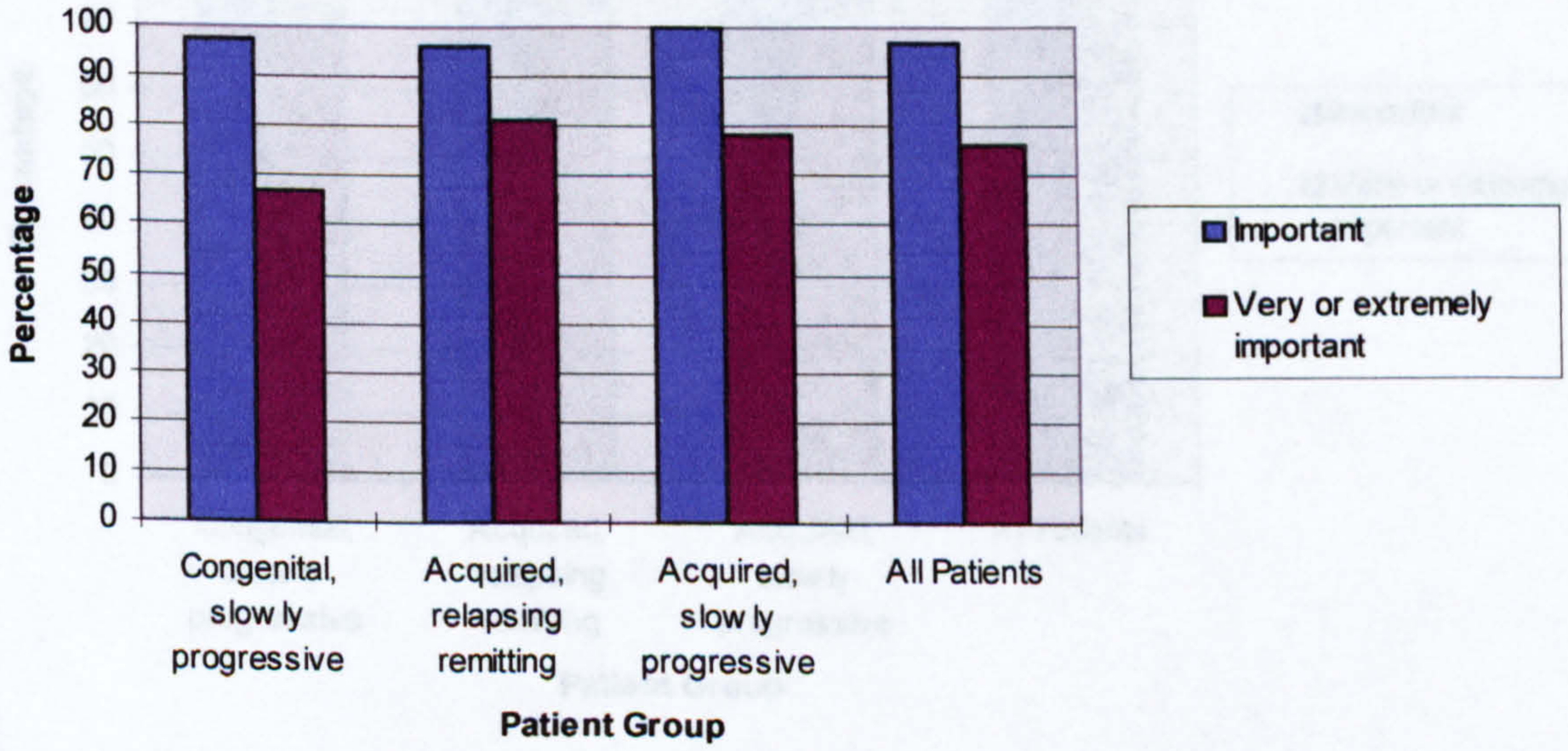


Figure 7.7: Impact upon working life (in those employed, n=142)

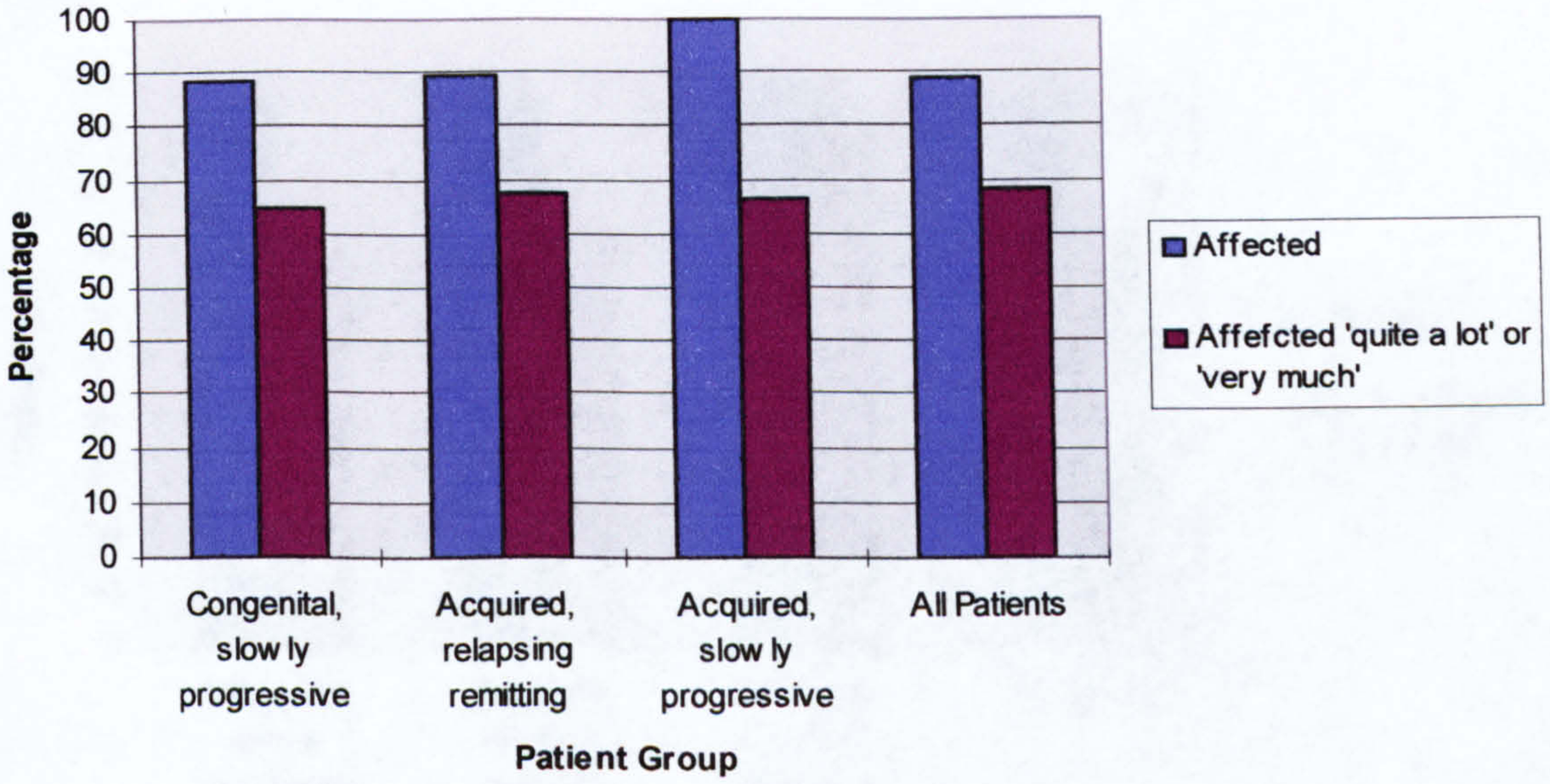


Figure 7.8: Importance of impact on working life as reported by survey respondents (in those employed, n=142)

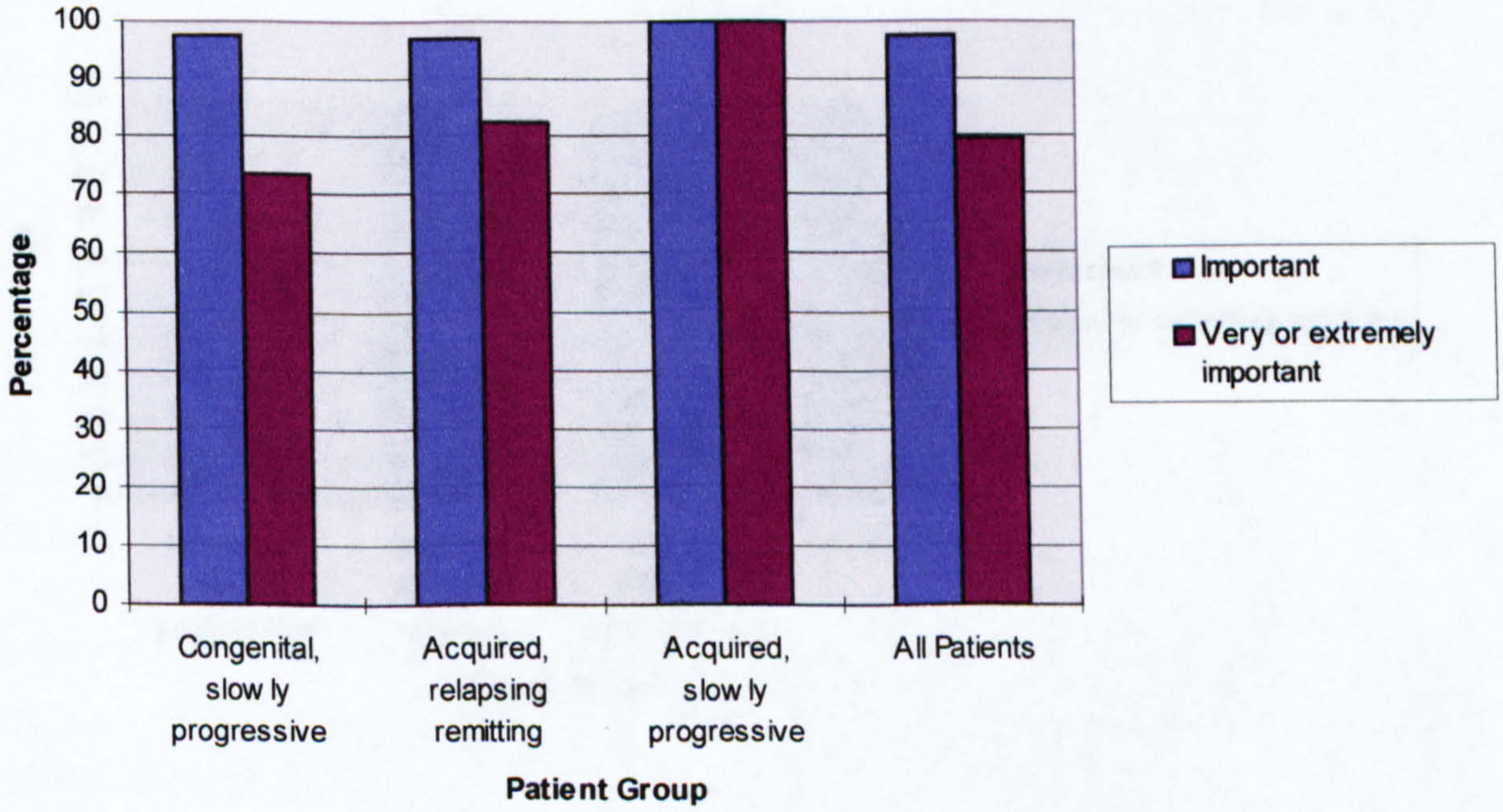


Figure 7.9: Impact on social and leisure activities

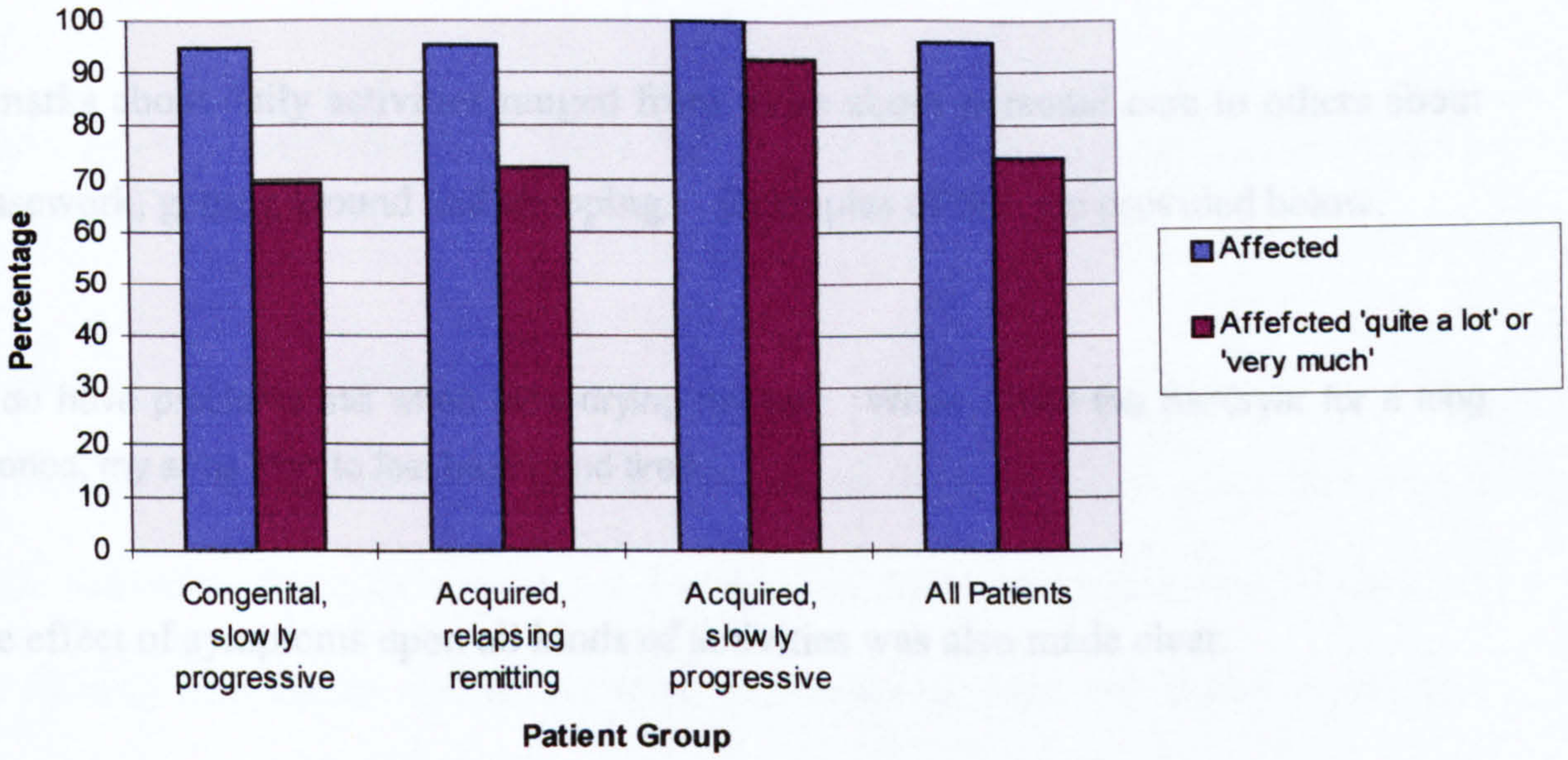
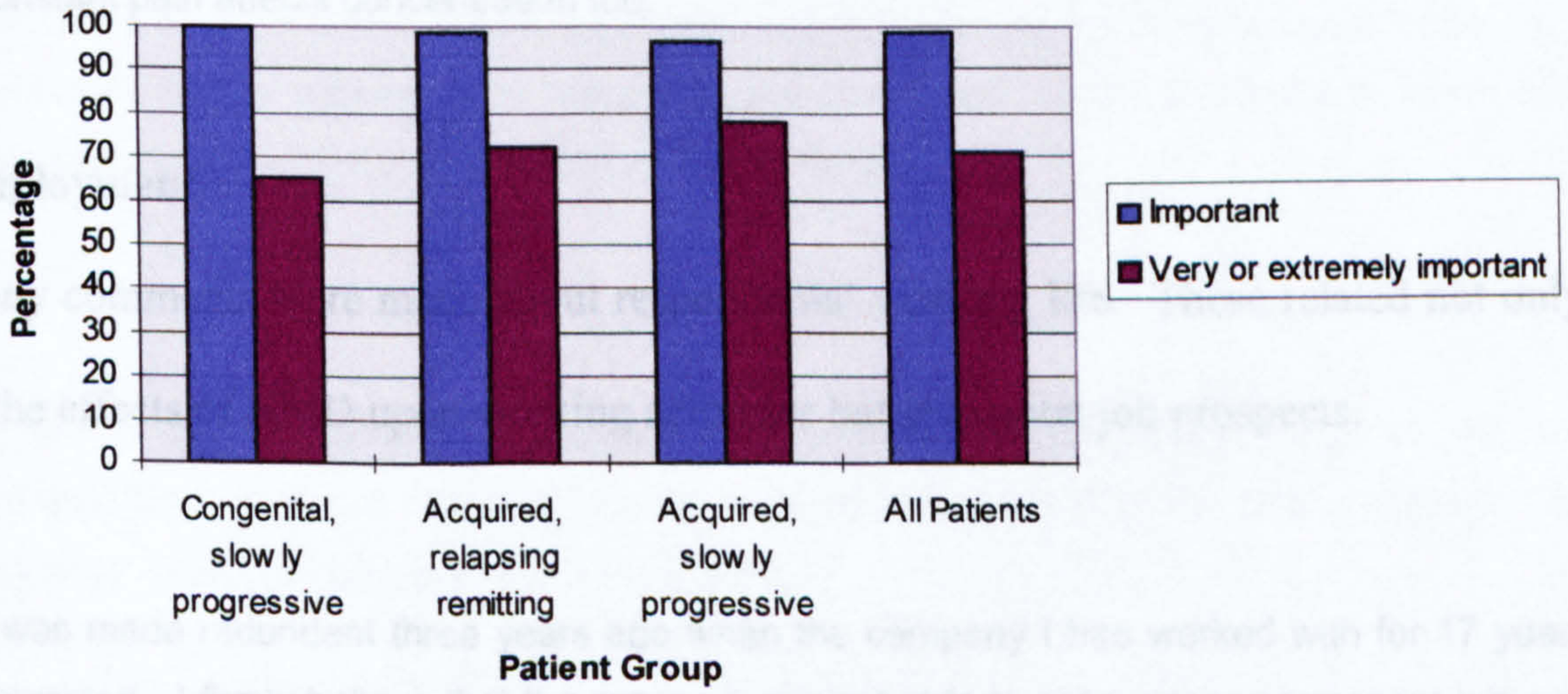


Figure 7.10: Importance of impact on social and leisure activities as reported by survey respondents



Daily activities

Respondents' comments tended to reiterate the experiences and concerns expressed by patients in the interview study.

Remarks about daily activities ranged from those about personal care to others about housework, getting around and shopping. Examples of this are provided below.

> I do have problems still when blow-drying my hair. When I hold the hairdryer for a long period, my arms start to feel heavy and tired.

The effect of symptoms upon all kinds of activities was also made clear.

> I do not have the strength/energy to sustain normal day to day living activities for more than a couple of hours. I become overwhelmingly tired and my muscles ache so much I cannot do anything or even concentrate on sedentary activities. This is despite painkillers- I only recover after lying down for a couple of hours. It takes so much effort to get through the basic needs of living that there is no energy/time left for social activities. The almost constant pain affects concentration too.

Employment

Many comments were made about respondents' working life. These related not only to the effects of NMD upon working activities but also upon job prospects.

> I was made redundant three years ago when the company I had worked with for 17 years relocated. I firmly believe that the reason I was not able to get another permanent job was because of my condition.

> This disability has prohibited me from achieving my full potential career-wise. I have been informed that I have been not considered for career moves on the grounds of physical limitations only.

Many respondents commented on their having to take early retirement on ill health grounds, or upon the adaptations they had made to their jobs or career path. The impact of stopping work upon self-identity was clear.

> Had to retire from work at age 35 – had a huge impact on how I saw myself as a contributing member of society.

Social and Leisure Activities

Respondents also commented upon the impact of their condition upon leisure and social activities. Both sporting and sedentary activities were influenced. Socialising and visiting with friends and family were commonly reported and linked to transport problems and difficulties in accessing buildings and homes.

> This illness affects all my leisure activities, as I'm in a wheelchair. I've had to give up dancing, gardening and even visiting friends and shopping. Even my son and daughter I can not visit as there are steps which I cannot get up over. Some shops have a ramp or are level but a lot aren't. I have been confined to the house for 5 months due to the fact I could no longer transfer from the car to my wheelchair. After 5 months of worry and expense we have just had a car converted for me to drive

> I used to be physically active with ice hockey + keep fit + badminton + squash. All now impossible. Sitting in theatres is often uncomfortable + stairs in theatres difficult. All outings, even car boot sales I find exhausting.

7.3.5 Relationships and Social interaction (questions 4-7)

The impact of NMD upon relationships (figures 7.11-7.18) was also very evident, with more than half of the respondents reporting an impact in all the relationship items. Nevertheless, the reported impact was not as elevated as it was across the activity items. Considerably fewer rated this impact as being 'quite a lot' or 'very

much', although more than a quarter of respondents still responded using these ratings. Relationship with partner/spouse (figures 7.11 & 7.12) was clearly the relationship influenced most by NMD and the one believed to be most important. Conversely, the 'not at all' option was most frequently selected for relationships with family (25%), with friends (31%) and in general social interaction (48%). Respondents in the congenital group also reported less impact and importance of impact in partner relationships (figs 7.11 & 7.12), family relationships (figs 7.13 & 7.14) and friendships (figures 7.15 & 7.16).

Qualitative Data

The themes emerging from the analysis of the comments made in the postal questionnaire were, again, similar to those that came out of the interviews.

Table 7.3: Relationship and social interaction themes emerging from postal survey comments

Domain	Subdomain	Specific Issues
Social impact	Partner	Meeting potential partner Strain on relationships Sex life Support from partner (*positive*)
	Family	Lack of support Worry about being a burden Worry about passing on gene Support from family (*positive*)
	Friends	Difficulty in visiting friends Loss of contact Support from friends (*positive*)
	General social interaction	Avoidance of situation Lack of understanding Difficulty explaining condition Other people's perceptions Problems of access Discrimination Encouragement of others (*positive*)

Figure 7.11: Impact upon relationship with partner/spouse as reported by survey respondents

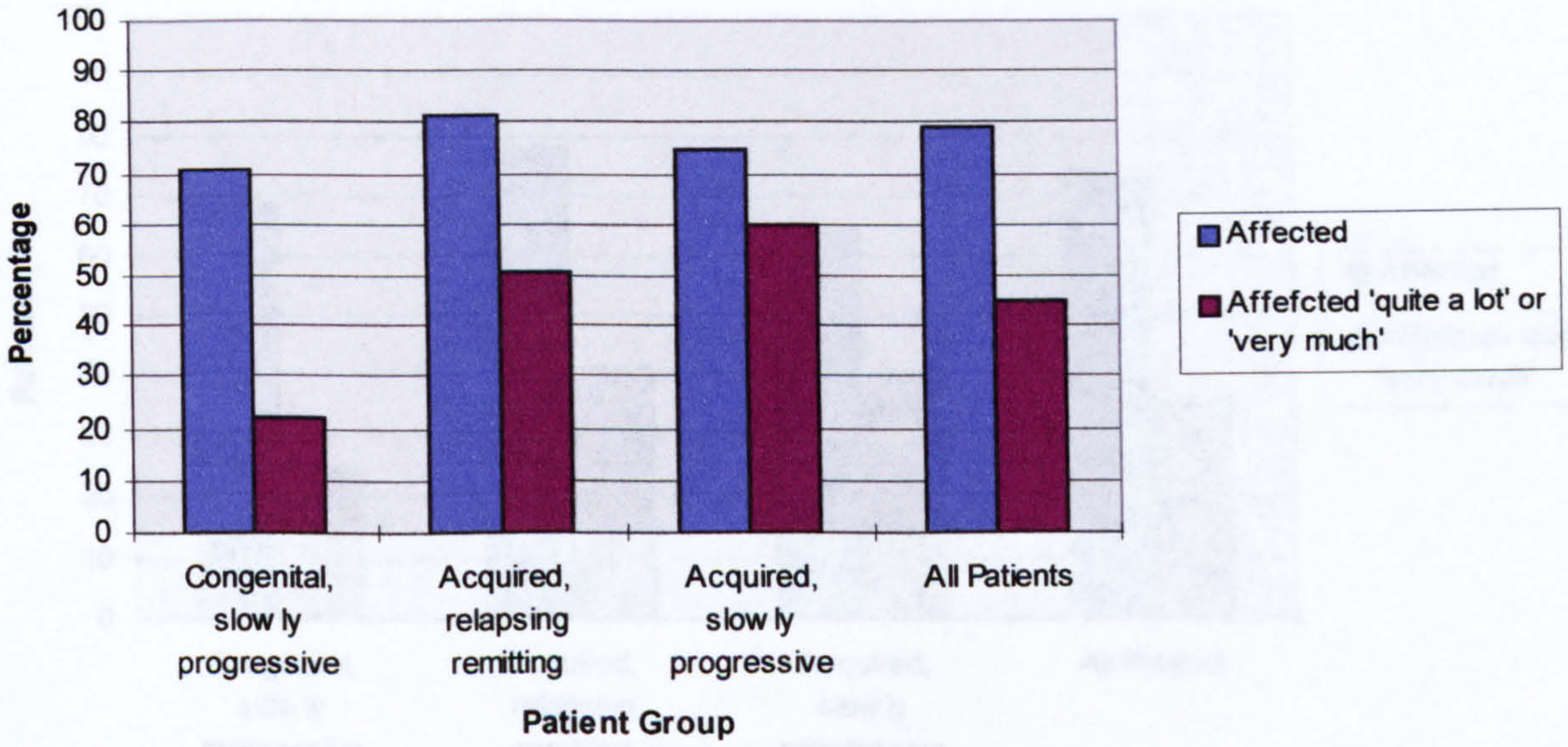


Figure 7.12: Importance of impact upon relationship with partner/spouse as reported by survey respondents

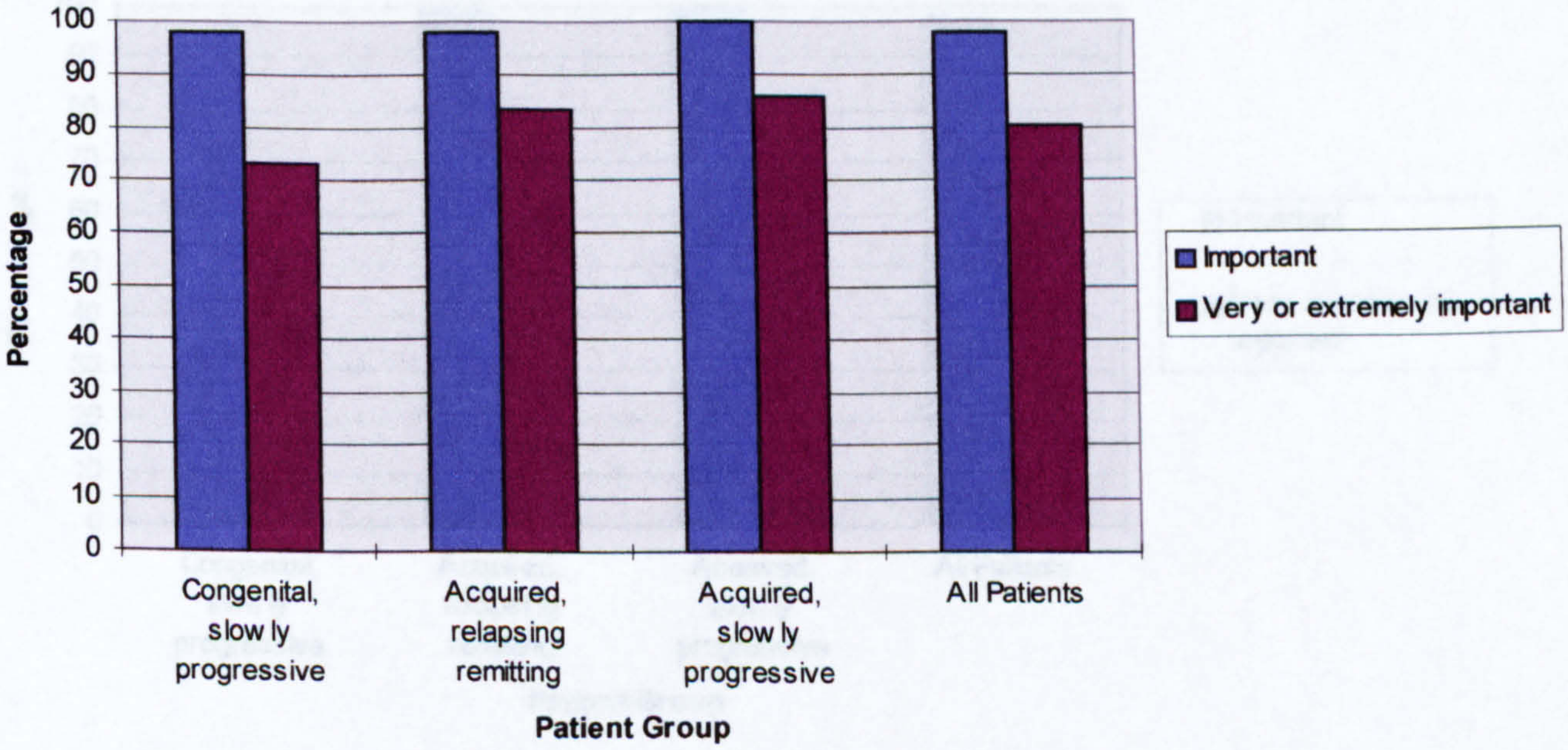


Figure 7.13: Impact upon relationships with family as reported by survey respondents

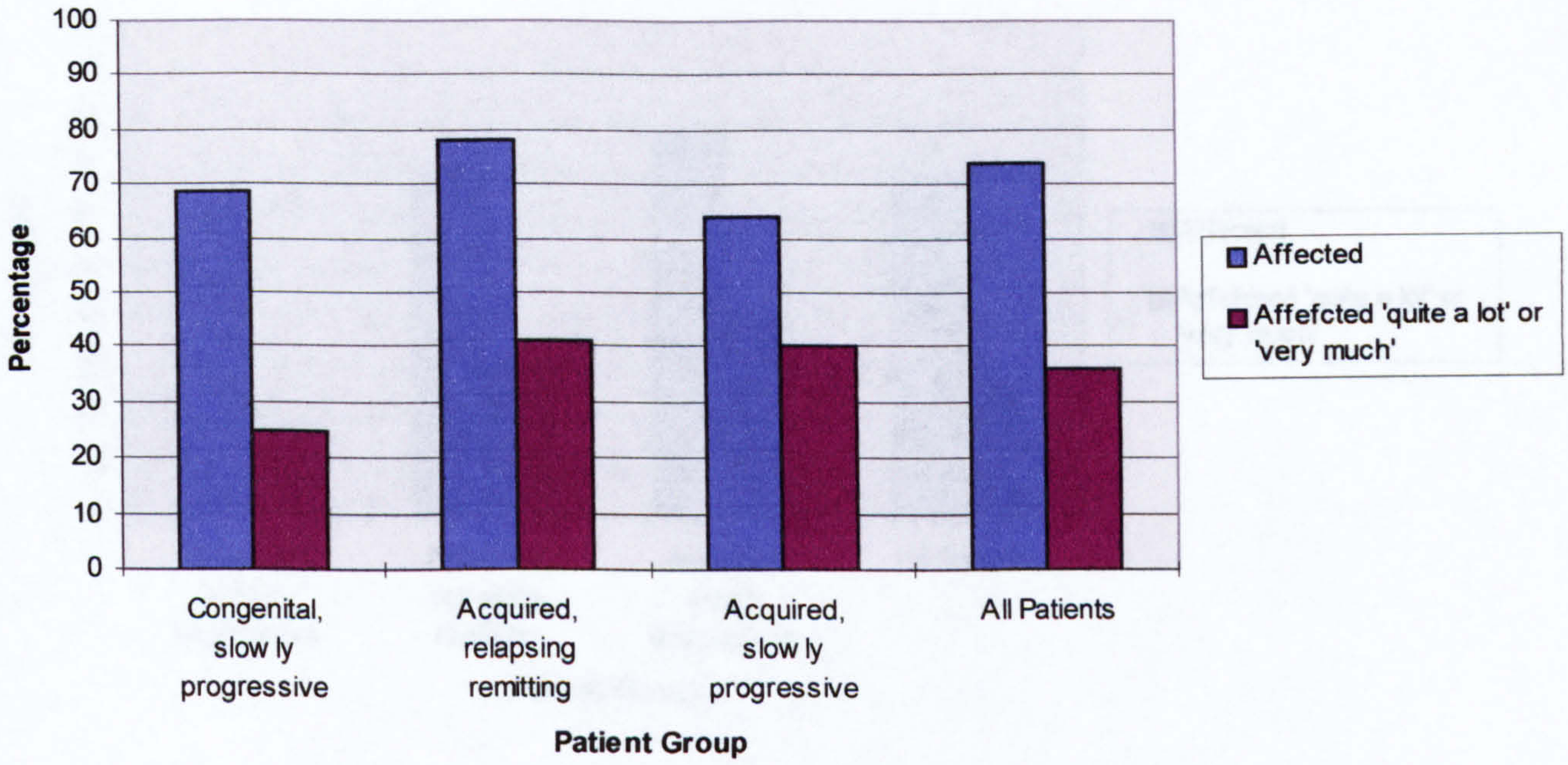


Figure 7.14: Importance of impact upon relationships with family as reported by survey respondents

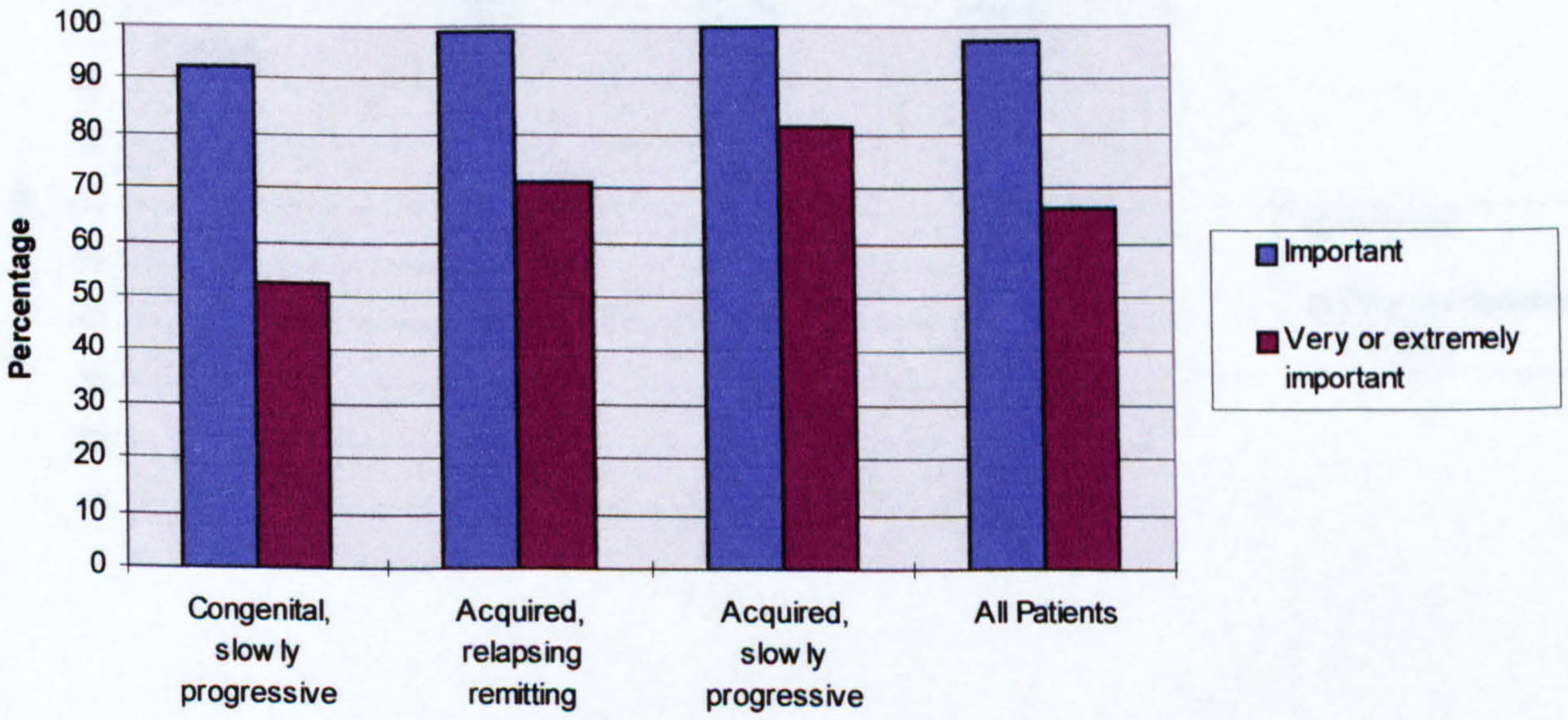


Figure 7.15: Impact upon relationships with friends as reported by survey respondents

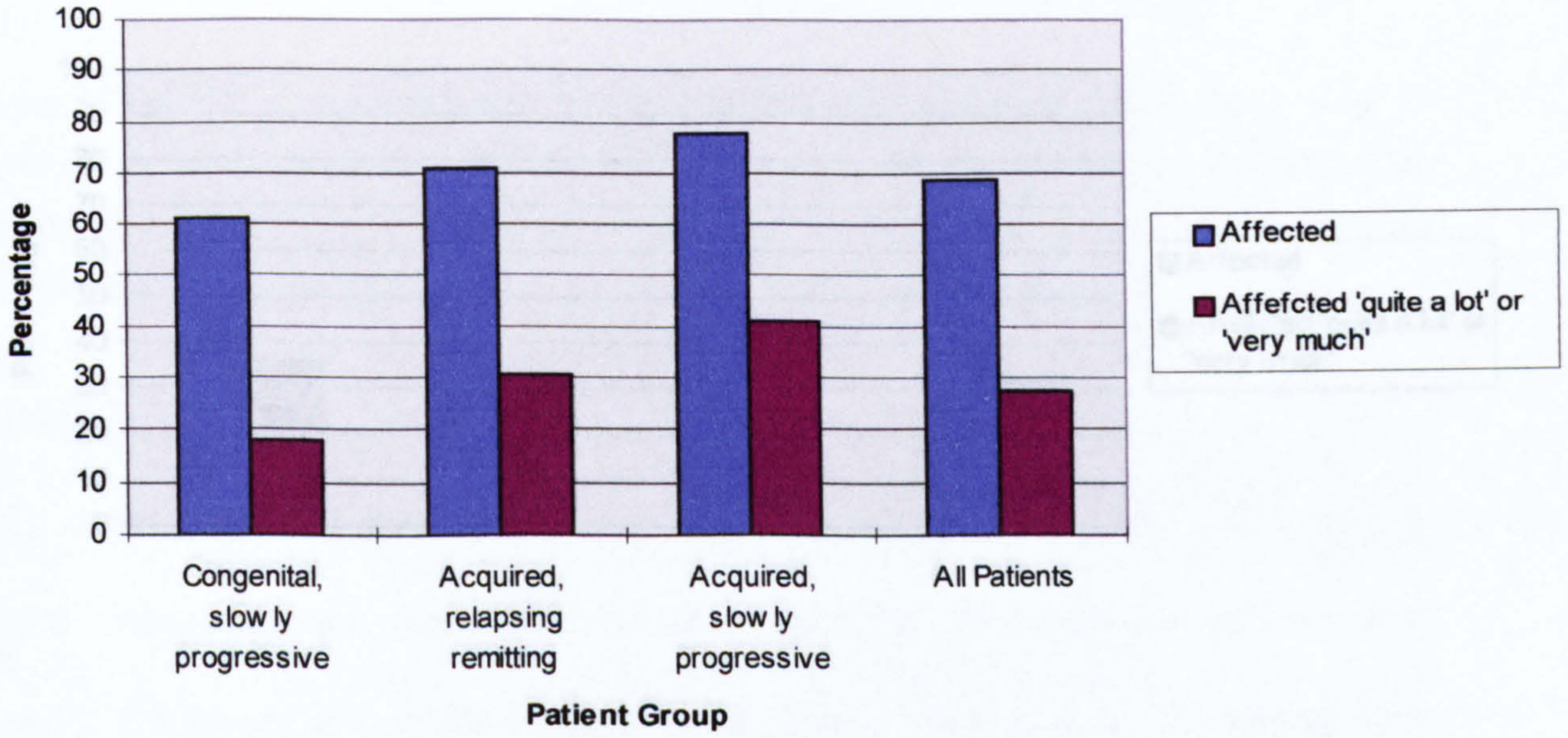


Figure 7.16: Importance of impact upon relationships with friends as reported by survey respondents

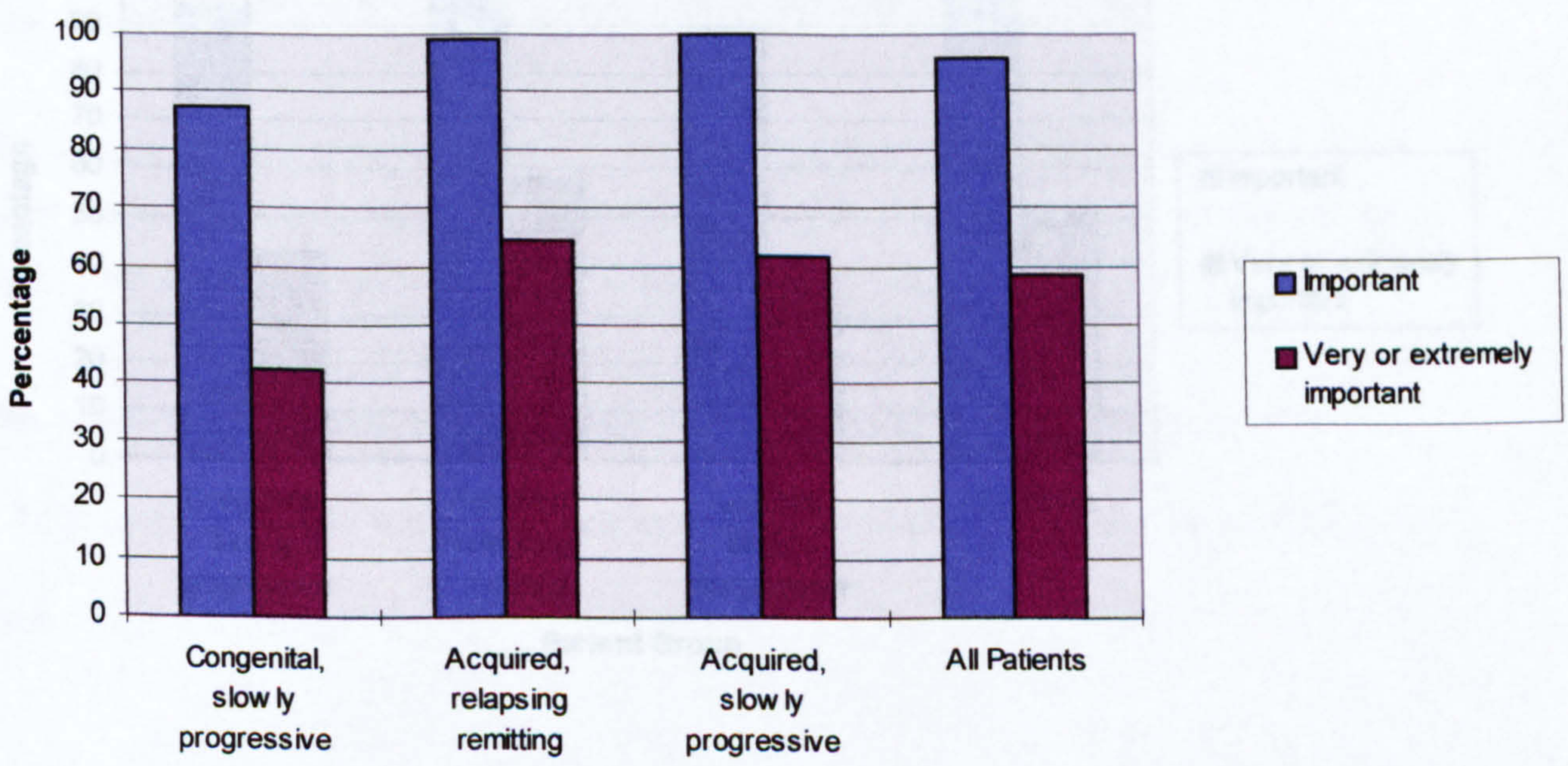


Figure 7.17: Impact upon general social interaction as reported by survey respondents

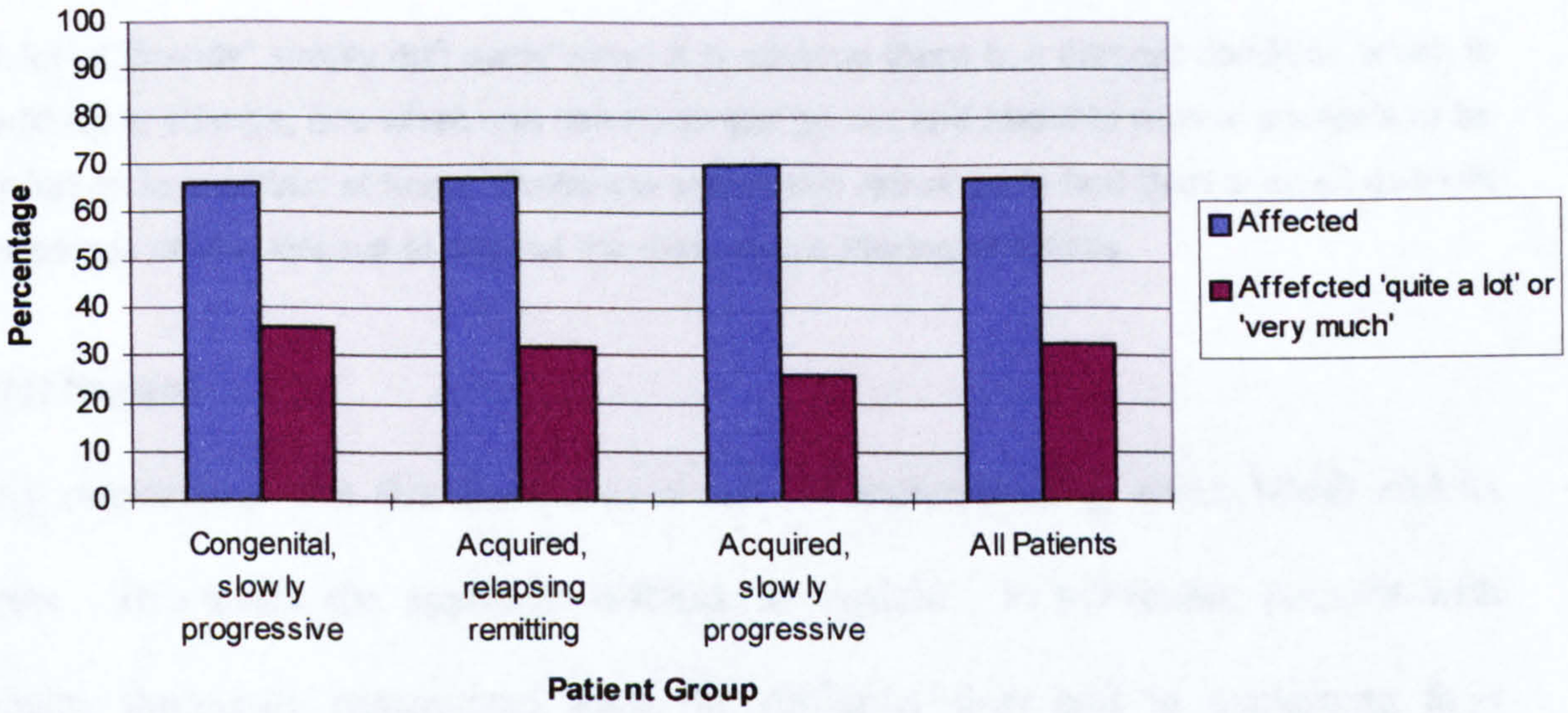
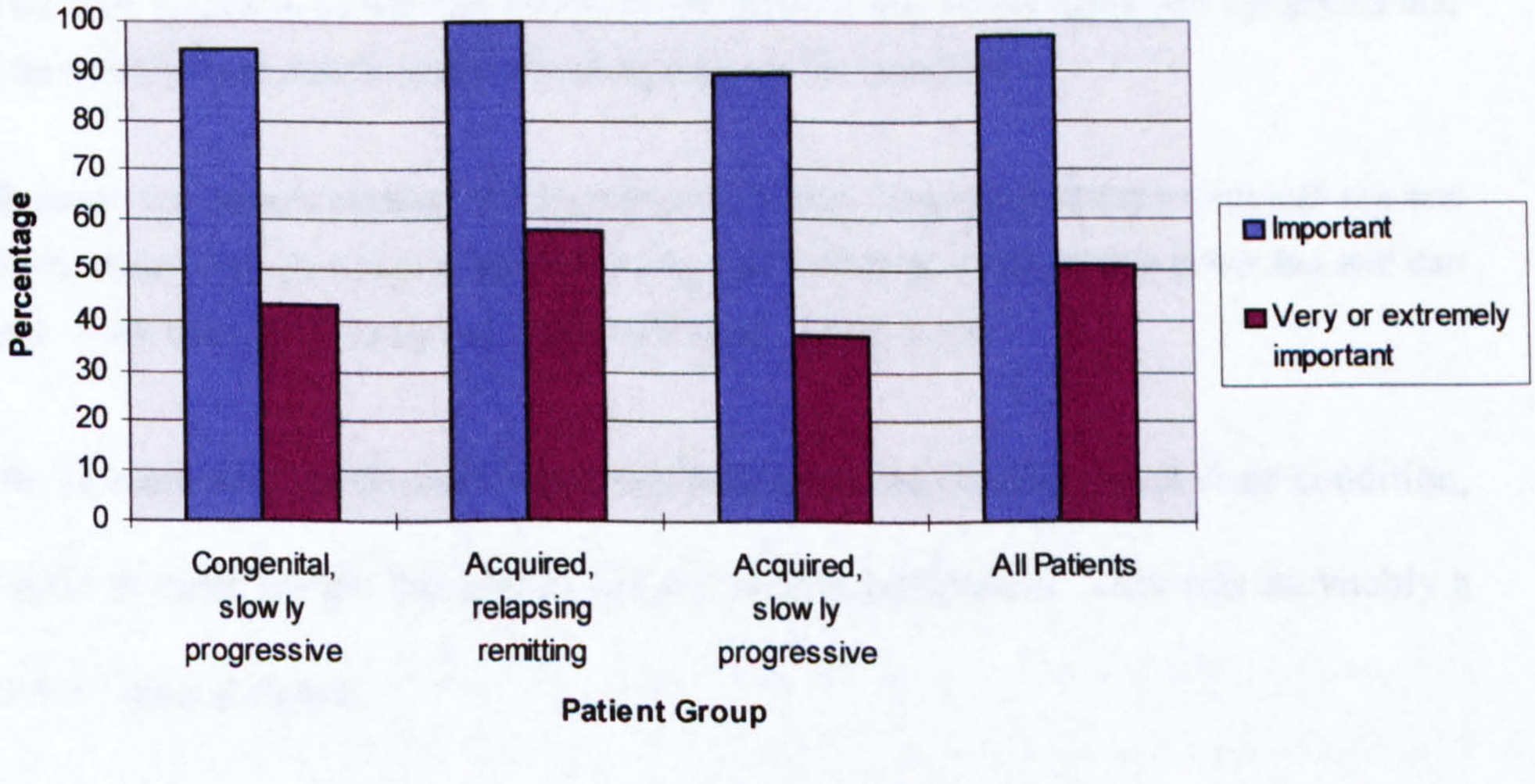


Figure 7.18: Importance of impact upon general social interaction as reported by survey respondents



Friends

Some respondents commented that friendships had faded as a result of difficulties taking part in activities previously shared with friends.

> A lot of "friends" simply drift away when it is obvious there is a chronic condition which is unlikely to change, and when one can no longer go out and about to mutual interests or be relied on to entertain at home. Someone who would rather go to bed than even sit and talk becomes of little interest to any but the most long suffering of friends.

Social impact

Many respondents felt that there was a lack of understanding about NMD and its effects. This made the condition difficult to explain. In particular, patients with Myositis frequently commented upon the difficulty they had in explaining their condition to other people. They tended to believe that this was because they looked well and therefore people didn't understand or empathise with their condition.

> The main problems lie with the condition not showing any visible signs and symptoms and often having to explain oneself or making excuses for inabilities.

> Because my muscle problem is not obvious to people they see nothing wrong with me and treat me accordingly which is very annoying and upsetting. Near friends know this and can see I walk badly and slowly and are sympathetic, which helps.

Some patients also commented upon the lack of understanding about their condition, not only in other people but also in the in medical profession. This was inevitably a source of great distress.

> Contact with other people and even my relatives, I cannot find a word to describe how terrible it is.
>
> 1. People cannot understand the muscle illness
> 2. They and also medical staff have never heard of "Inclusion Body Myositis"
> 3. They cannot understand why I can't have an operation.
> 4. They cannot understand why I take no medication

5. They see me in Asda in my wheelchair and say "you look well", little do they know. I feel fatigued, lethargic, utterly tired out all the time, and there is nothing and nobody out there who can help me. I feel very isolated and very much alone with this miserable illness

Family

An immediate effect upon some respondents' behaviour towards their family was evident.

I find myself distancing myself from my family. If I am too caring I may be expected to physically do something to show that I love them. I used to do it in the past, even though I suffered for it- their needs were met whilst I cried alone in pain. I'm continually trying to balance my relationships but this year have felt, I just want to be left alone so that I can at least cope and have enough energy to be reasonably cheerful and good company for my husband who I am sure you appreciate has had a lot to cope with himself. To be honest I don't answer the phone or the door while my husband is at work and when he comes home I let him do it.

Problems of access to public facilities and the homes of friends and family were also a source of difficulty (examples in 'Social and Leisure Activities' section).

Partner

A large proportion of the respondents commented upon the impact their condition had exerted upon relationship with their spouse or partner.

My muscle condition, weakness + tiredness destroyed my marriage in the end. I'm now on my own and can see no future with myself ever having a relationship with anyone ever again, it's just too hard.

We don't share activities any more, we can't go on same kind of holidays we used to, I'm a nuisance when we go out together, and I've gone off sex completely. I can't see why he still bothers with me.

A number of respondents also commented upon the difficulties they had in meeting potential partners, an issue that was not explicitly mentioned in the qualitative interview study.

> When it comes to a partner, everything is fine until I mention my illness, in which case they don't want to know.

However, the support provided respondents' families and partners was also clear from a number of comments.

> When I was finally diagnosed my fiancé and friends were really supportive and helped me to get through the most difficult times.

7.3.6 Psychological Impact (questions 8-11)

Patients were most commonly affected 'very much' by their muscle condition across the psychological impact items, and most frequently rated this impact as 'extremely important' (figs 7.19-7.26). The psychological items gained higher ratings of impact than did the relationship items. However, compared to the activity items they received slightly fewer ratings at the high end of the scale.

Responses to the independence item showed this issue to be of considerable importance to all patients with well over three-quarters of respondents reporting the impact upon their independence as being either 'very' or 'extremely important' (fig 7.20). Similarly, the importance attached by patients to their perceptions of the future was also very high (fig 7.26).

From the data, it was clear that many patients believed their physical appearance to be negatively affected by their muscle condition (figs 7.23 & 7.24). Nevertheless, body image received lower impact and importance ratings in patients with an ASP condition.

Figure 7.19: Impact upon independence as reported by survey respondents

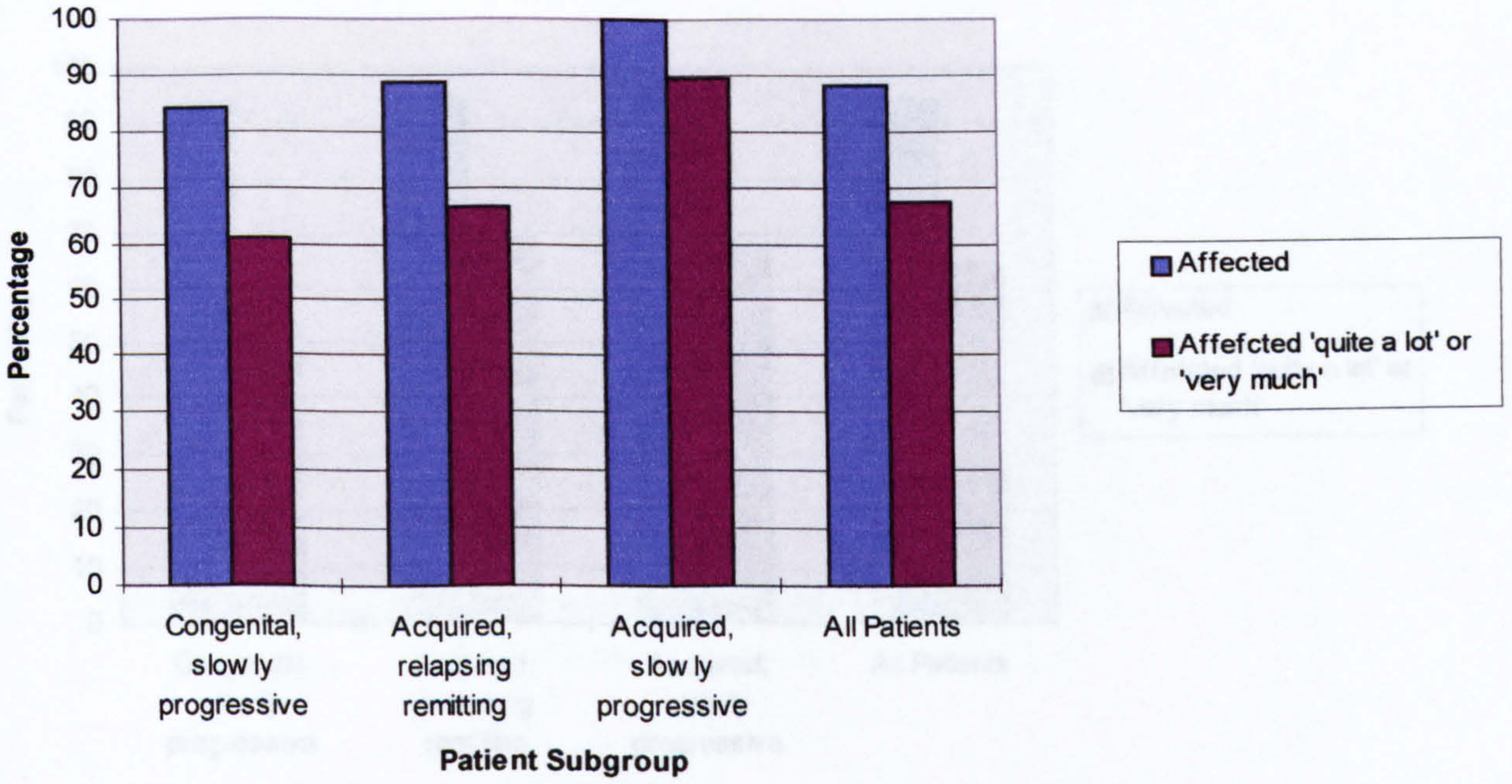


Figure 7.20: Importance of impact upon independence as reported by survey respondents

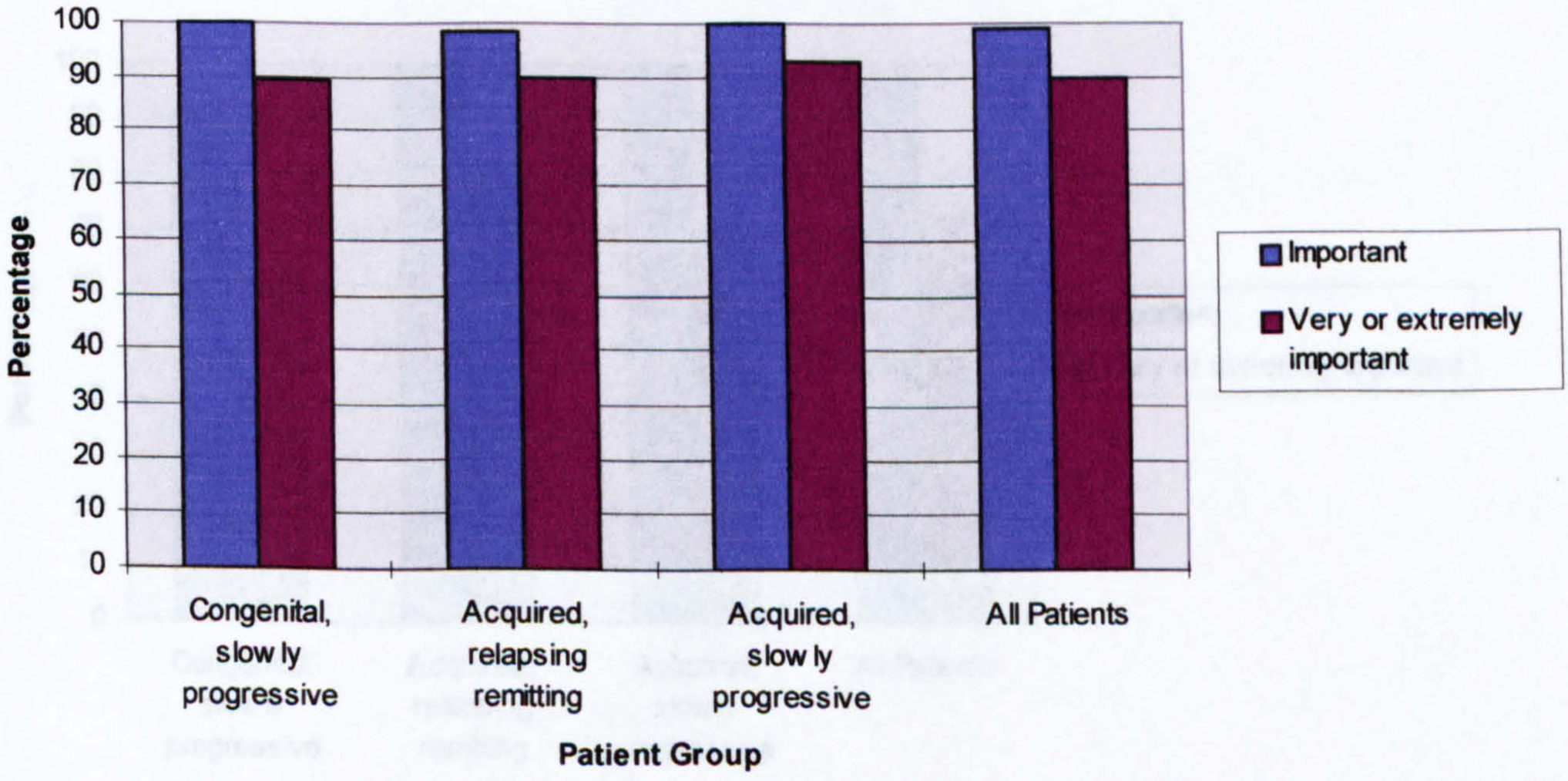


Figure 7.21: Impact upon emotions as reported by survey respondents

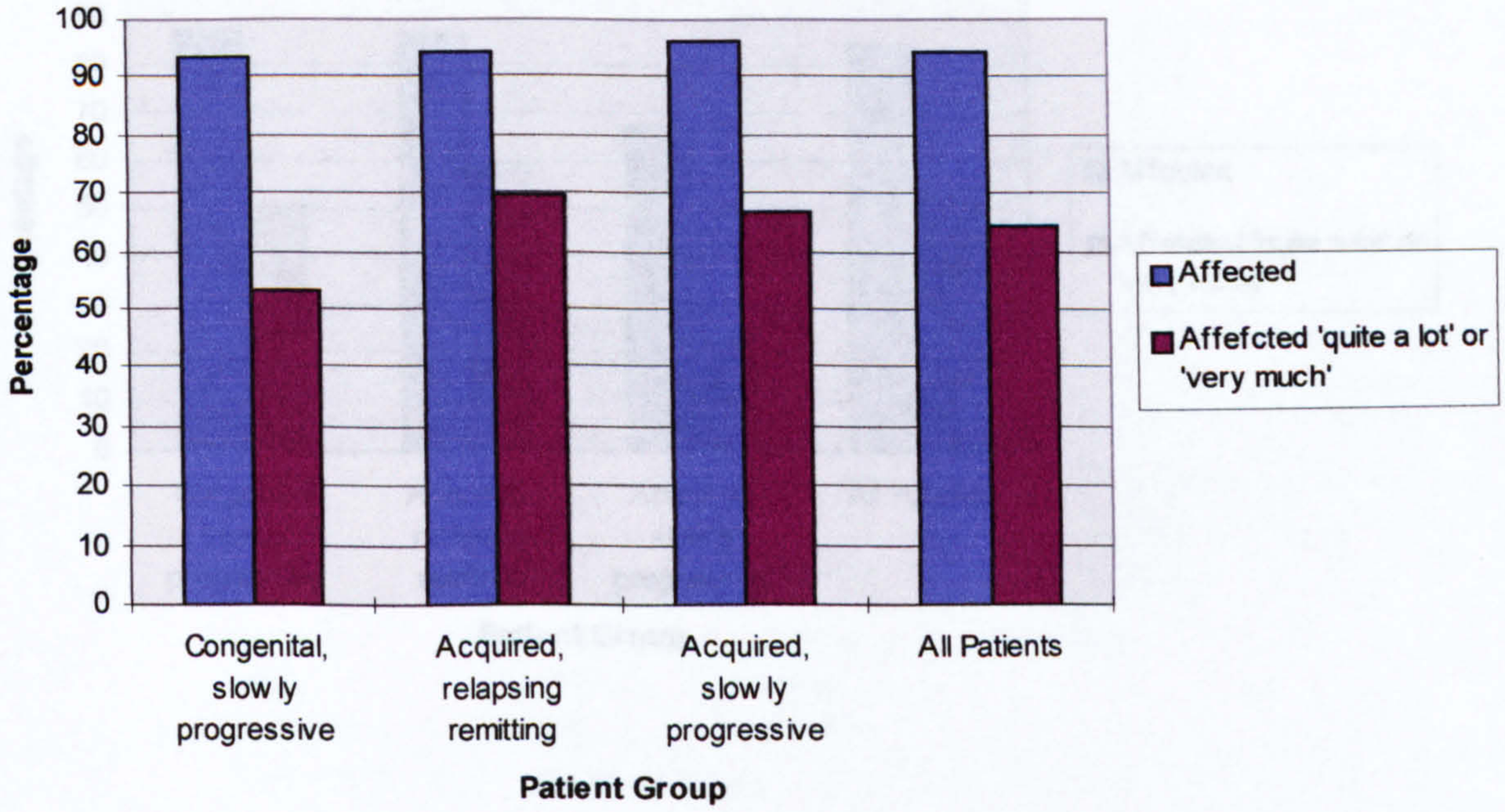


Figure 7.22: Importance of impact upon emotions as reported by survey respondents

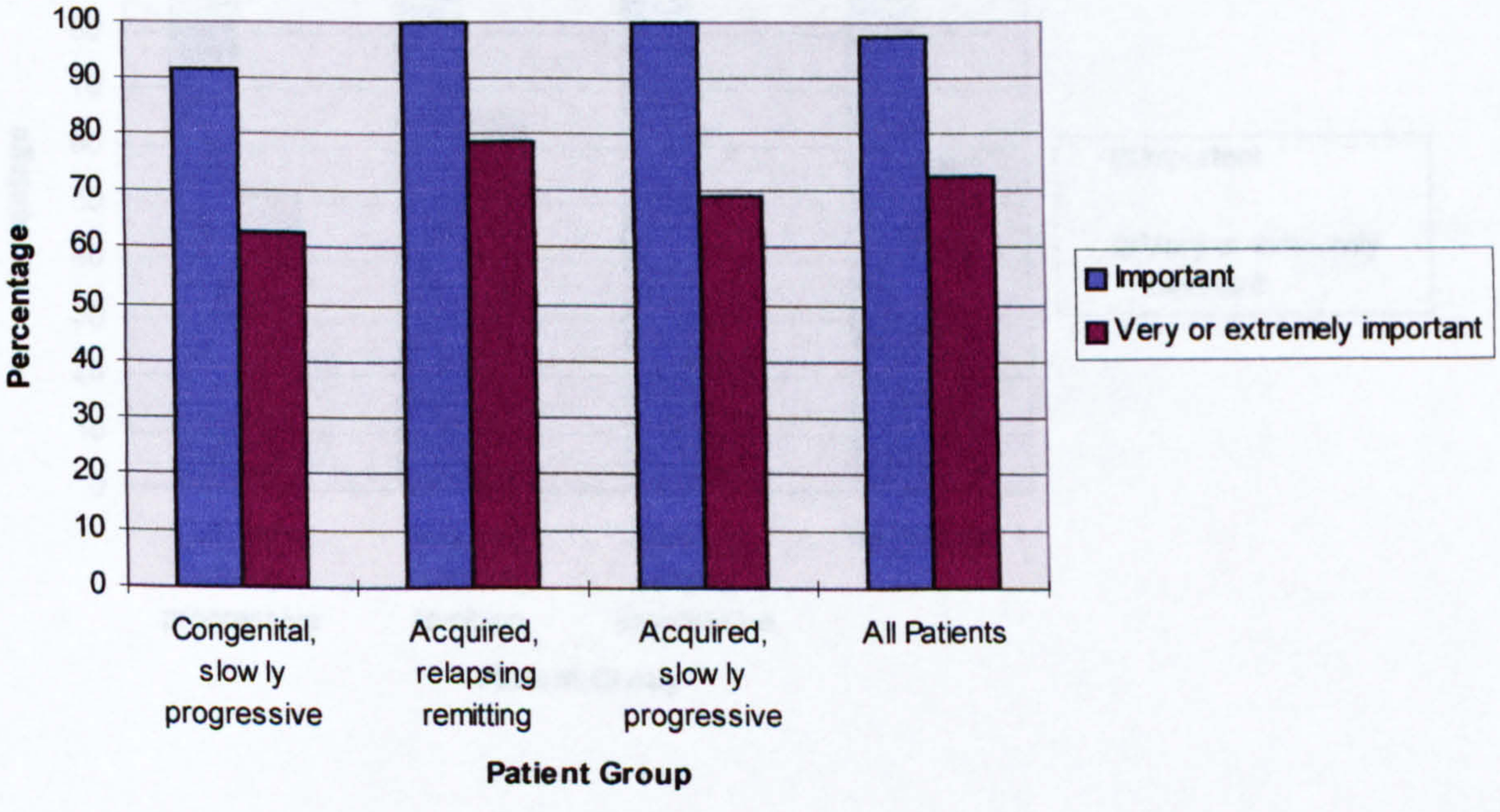


Figure 7.23: Impact upon body image as reported by survey respondents

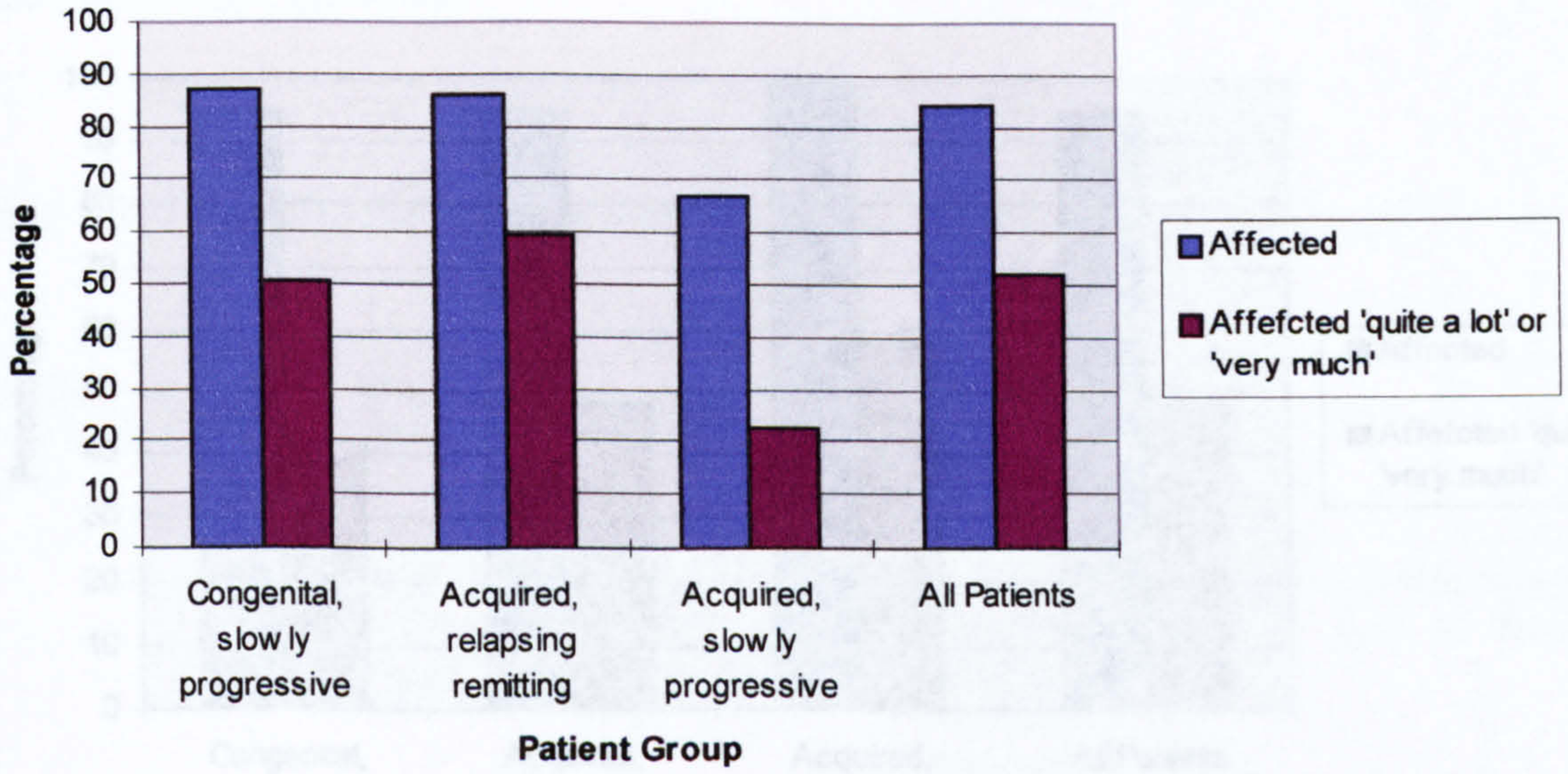


Figure 7.24: Importance of impact upon body image as reported by survey respondents

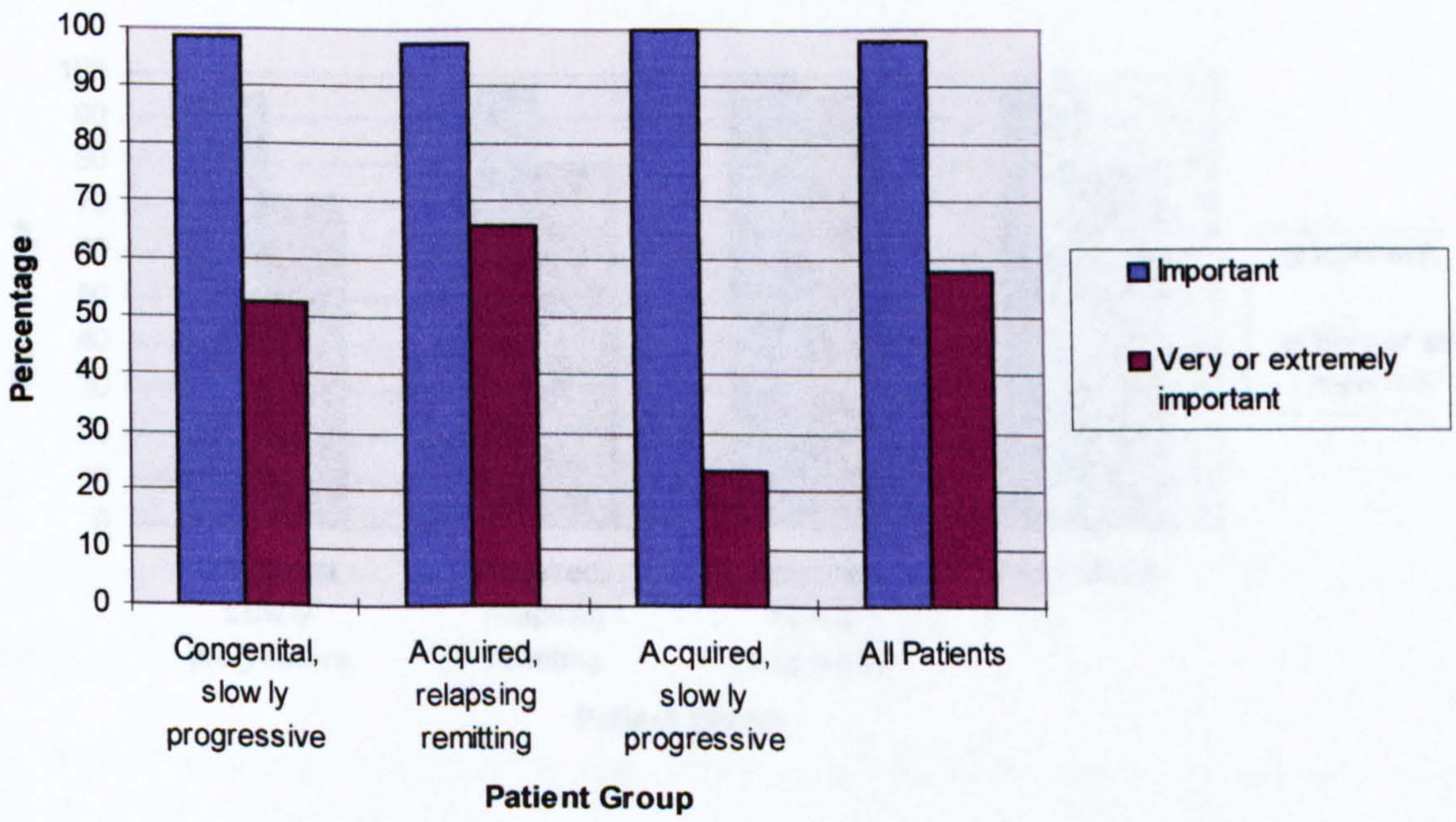


Figure 7.25: Impact upon perception of the future as reported by survey respondents

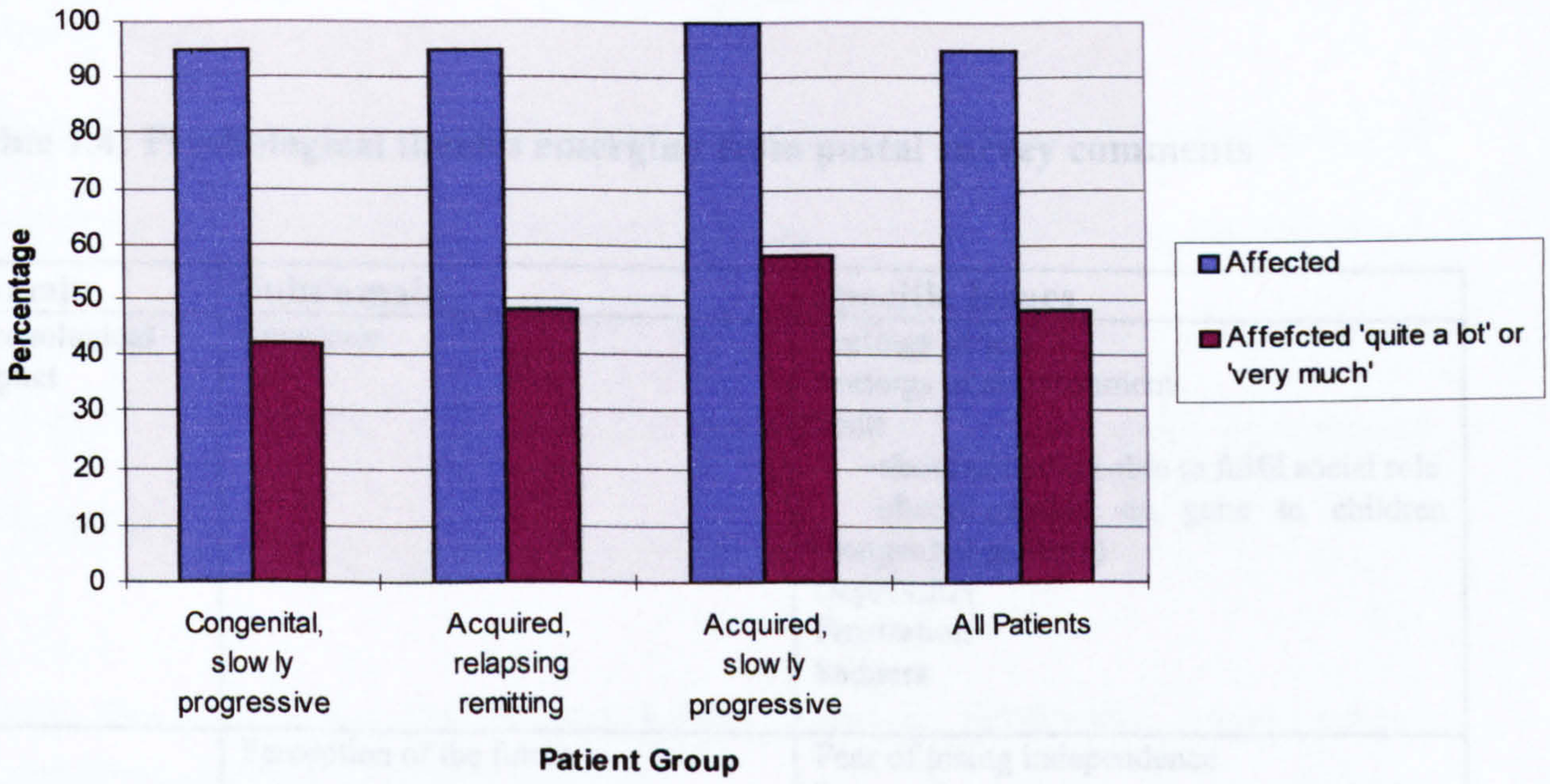
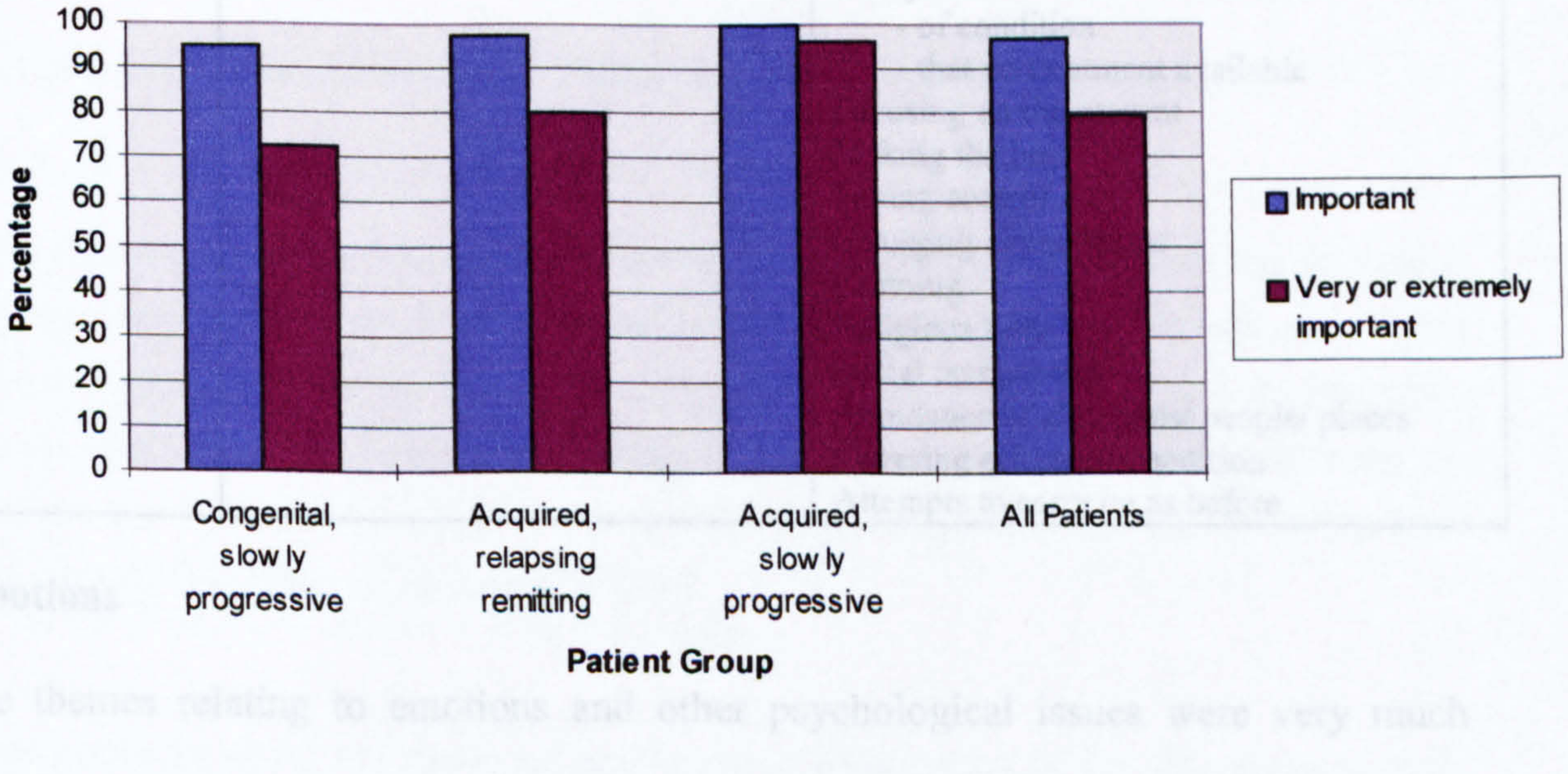


Figure 7.26: Importance of impact upon perception of the future as reported by survey respondents



Qualitative Data

Once again the emerging categories bore similarities to issues that emerged from the semi-structured interviews.

Table 7.4: Psychological themes emerging from postal survey comments

Domain	Subdomain	Specific Issues
Psychological Impact	Emotions	Feelings of loss Feelings of abandonment Guilt -about not being able to fulfil social role -about passing on gene to children (congenital patients) Depression Frustration Sadness
	Perception of the future	Fear of losing independence Fear of passing on gene to children (congenital patients)
	Identity / Self-image	Body image Social image/identity/role
	Independence	Loss of control
	Coping strategies	Determination Acceptance - of condition - that no treatment available Focusing on the present Making the best Taking control Changing expectations Planning Religious faith Social comparison Avoidance of situations/ people/ places Covering effects of condition Attempts to carry on as before

Emotions

The themes relating to emotions and other psychological issues were very much intertwined and respondents tended to comment upon the emotional impact of NMD in the context of other life domains. For example, patients' fears about the future had a considerable impact upon the emotional feelings expressed.

> Sometimes I feel well balanced and happy and yet other times I feel sad and lonely even though I have a wonderful family. I picture my future as being on my own, ill and more dependent than I am now, but I try not to think about the future.

Independence

Independence was very frequently commented upon in the survey. Frustration at being dependent upon others to carry out activities and fears of becoming dependent or more dependent upon close family members were prevalent. These themes overlapped considerably with those relating to impact upon family and partner relationships.

> I am very dependent on my husband and this affects me emotionally. I feel a burden sometimes, although he doesn't complain.

Perception of the Future

Perception of the future was frequently commented upon and was closely linked with fears of losing independence and of becoming a burden to family members.

> My thoughts about the future are very fearful. I know this illness is supposed to be slow in progressing, but how slow? At the moment I can only stand for a few seconds and if I dare bend my knees I'm on the floor in one untidy heap and can only get up with my husbands help, with a hoist. So I cannot walk or stand. I cannot lift my head off the pillow when I'm in bed and its very difficult to turn over in bed... what happens if I get worse. My husband is seventy-five. What will happen if he dies?

Other concerns about the future related to the genetic nature of the congenital muscle conditions. A number of respondents were worried about the possibility that they had passed on their condition to their children. They also had concerns about being able to physically care for their children in the future.

I tend to worry a lot about how much of a burden I will become to my family, my wife especially, as I get more immobile. Also I worry a great deal about what I have passed onto my daughter. They learnt about genetics at school, so they know that it is passed on, but I was told by a doctor at an FSH get together not to have them tested unless it was absolutely necessary as the tests were not always conclusive.

I worry about the future. Especially whether I have passed on my condition to my son. I worry that I will not be strong enough to carry him when he gets heavier

Self Image, Identity and Body Image

Loss of independence had a considerable impact upon patients' self-image and sense of identity. This impact upon self-image related to the effects on NMD upon social role fulfilment

I don't really have any independence. I think I must have lost the will to even want to be independent. I don't feel like I'm a person any more- maybe just an extension of my husband most of the time, which is not so bad because he's a really nice guy!

Effects (of NMD and steroid treatment) upon patients' physical appearance also had implications for patients' self-image and confidence.

I hate the way long-term steroid medication has made me look. I cannot bear to look in a mirror and I just cannot look at photos of myself taken before my condition.

The gradual erosion of the physical being that I am is not a nice thing to contend with. It has a debilitating effect on your general persona.

This impact upon body image also had implications for social and sexual confidence.

I'm very self-conscious when it comes to the sex side of the relationship and I want to hide myself as much as possible. Consequently I find myself "putting off" the physical side of the relationship and so not encourage it even though we both still enjoy it when it does

happen. I'm a bit overweight because of lack of exercise so I am trying to cut down my intake of food.

Coping

Despite the impact of NMD on the outlook of many patients, determination and optimism were apparent in a number of reports.

I am optimistic about the future, which will involve study, more sedentary hobbies and refusing to be beaten by this disease.

The desire to find fresh challenges when my sporting life ended prompted me to take further study. This has proved very valuable in terms of personal development & improvement of self-esteem. I made many new friends and learned there is life after disability. I know this has encouraged others.

Coping strategies were an important issue in responses given by patients.

The future frightens me, so I have trained myself not to think too far ahead and to enjoy what independence I have today.

I try to be grateful for the things I can do and take each day as it comes.

Religious faith was another way in which some respondents coped with their condition and maintained a positive outlook

As I believe in life hereafter, I look forward to being without my physical handicaps... I think!

Other methods adopted by patients to minimise the possibility of negative outcomes included:

a) Planning ahead

Because of the condition it is not easy to do things on the spur of the moment. It is not always practical to go to places that are new to me without a bit of forward planning. For example, are there stairs or lifts, how far is it to walk, what is the ground like?

b) Comparing their situation to others they believed to be less fortunate,

After reading what other folk have gone through I feel very fortunate to be as healthy as I am.

c) Changing their expectations.

One has to accept that there are many things one can no longer do- if these are very important one has to ask for help- if not the there is a conscious decision to do without or accept a lowering of expectations and a compromise on previously acceptable standards.

7.3.7 The effects of treatment

In those receiving treatment, most respondents reported experiencing 'quite a lot' of beneficial effects and 'some' negative effects. Figure 7.27 indicates respondents reported experiencing roughly equal amounts of beneficial effects and side effects. Both the good and the bad effects of treatment were also most commonly rated as being 'extremely important'. Figure 7.28 demonstrates that positive treatment effects generally received higher importance ratings.

Most of those not receiving treatment (mainly patients with a congenital NMD or an ASP NMD) stated that this was because there was no treatment available for their muscle condition (figure 7.29). Most of the patients who had stopped treatment because of side effects were myositis patients.

Figure 7.27: Patients reporting beneficial effects and side effects of treatment (n= 124)

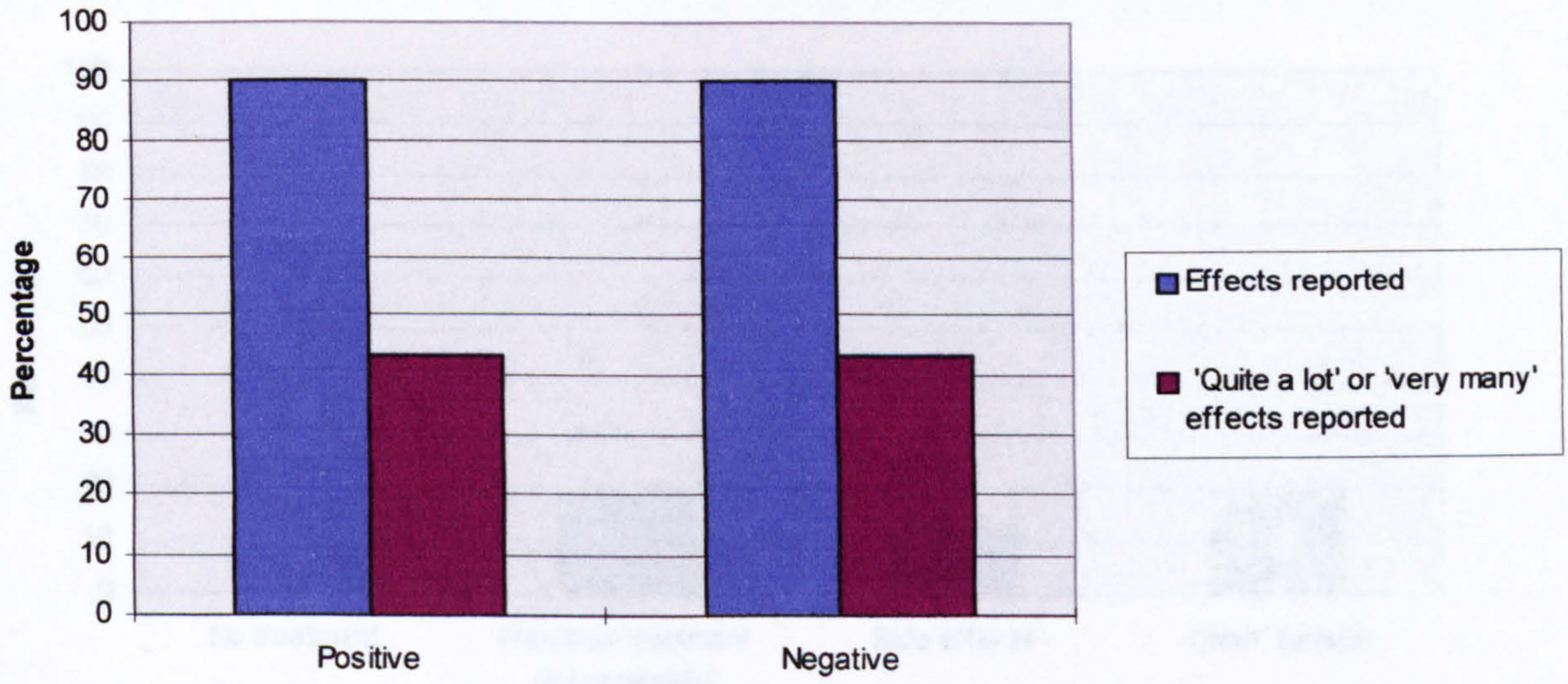


Figure 7.28: Patients reporting treatment effects as important

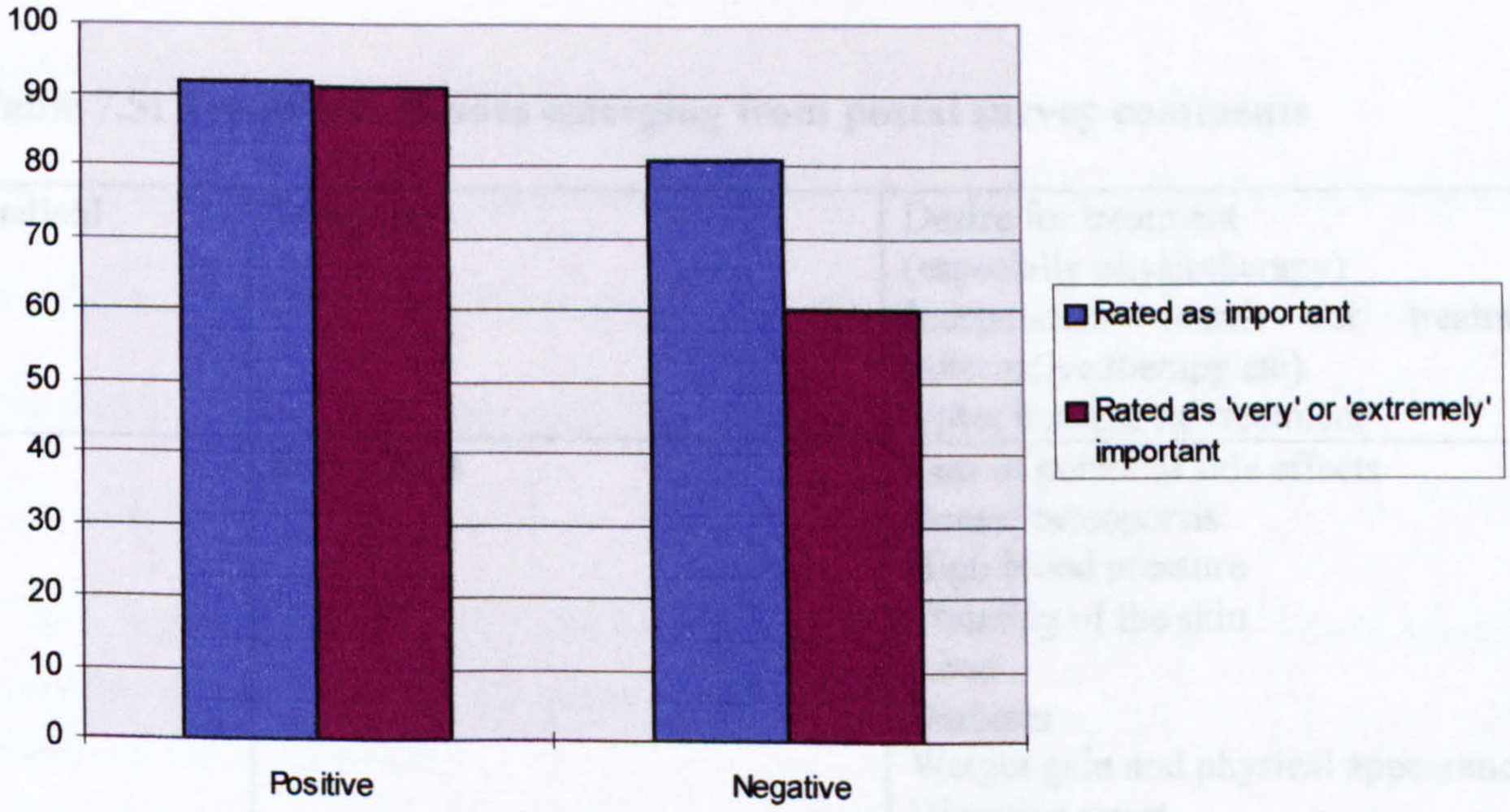
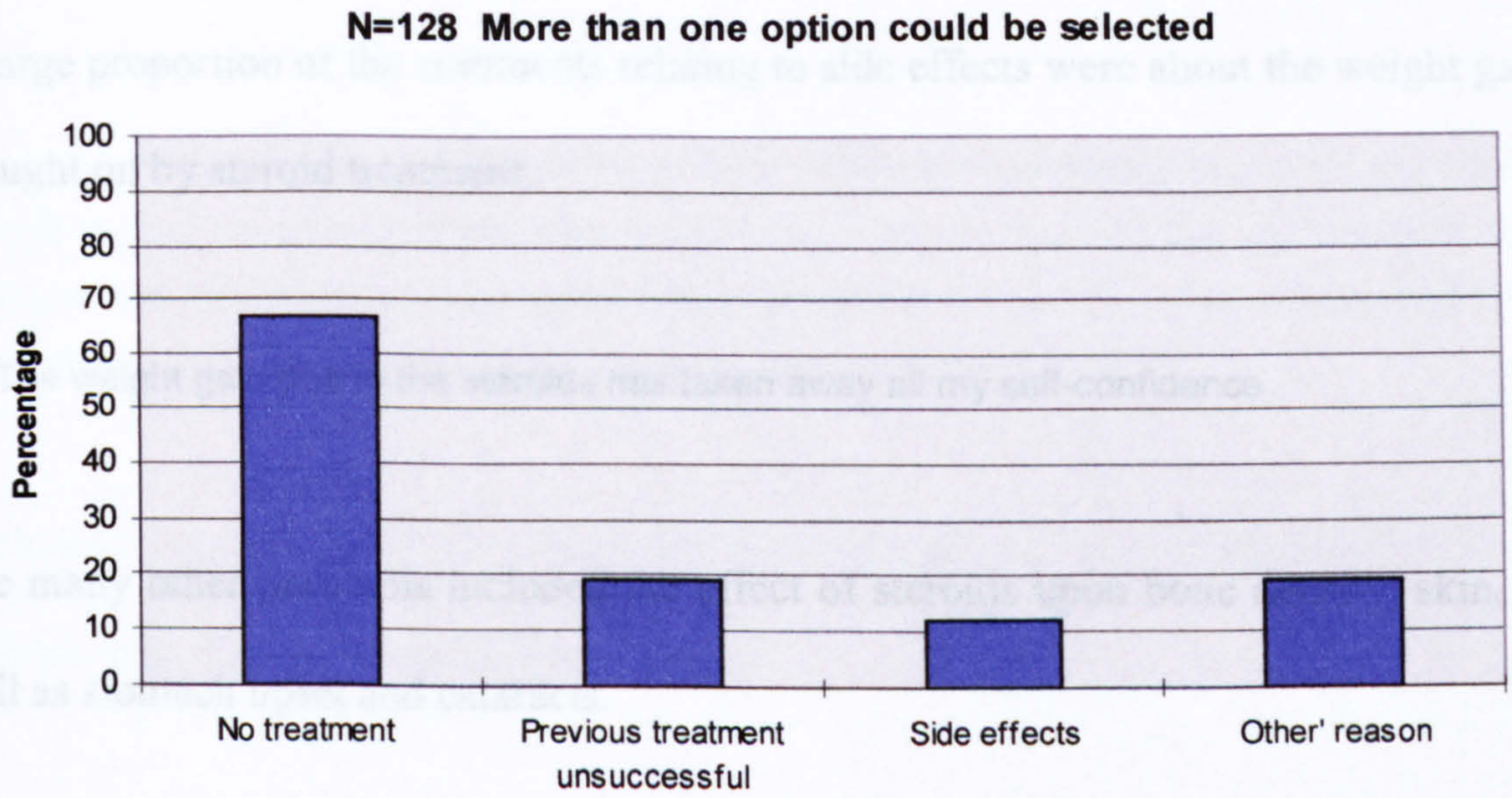


Figure 7.29: Reasons given for not receiving treatment in those patients not taking medication



Qualitative analysis

The postal questionnaire provided a section in which comments could be written about how patients felt about their treatment or lack thereof.

Table 7.5: Treatment themes emerging from postal survey comments

Medical	Treatment	Desire for treatment (especially physiotherapy) Independent search for treatment (alternative therapy etc) Upset because no treatment
	Side effects	Fear of potential side effects Bones/ osteoporsis High blood pressure Thinning of the skin Acne Diabetes Weight gain and physical appearance Digestive upset Cataracts Emotions/mood upset
	Feelings about care	Feelings of abandonment Inadequate care/support (of medical/ social nature)

Almost all the patients receiving treatment reported side effects. Most of these were due to steroid treatment.

A large proportion of the comments relating to side effects were about the weight gain brought on by steroid treatment.

> The weight gain due to the steroids has taken away all my self-confidence

The many other problems included the effect of steroids upon bone density, skin, as well as stomach upset and cataracts.

> My bone density has become low so have to an osteoporosis therapy. My skin has thinned considerably - cannot go out in the sun. Take a tablet for stomach problems and have developed sinus troubles. I notice I appear to be more anxious on treatment. I know the treatment has caused more muscle wastage. Have developed cataracts in eyes (optician says due to treatment). Positive aspect: - treatment makes life bearable.

7.3.8 Complementary medicine

Many patients commented upon treatment and therapies they had sought outside conventional medicine. The complementary regimens included acupuncture, homeopathy, special diets and dietary supplements, hydrotherapy and massage.

> There doesn't seem to be any kind of treatment from specialists or GPs. I have special, very light massage and exercises in a hydrotherapy pool
> (Meir Schneider technique)

> My treatment has been homeopathy, acupuncture and I believe the one most important factors has been sticking to a healthy diet and taking supplements.

Some patients felt there was a lack of support from medical and social services. Patients linked their feelings of being abandoned by the medical profession to the lack of treatment available for, and limited knowledge about their condition.

> I feel I don't get too much help from doctors. They are supportive to a point but cannot help to treat me and (as I have been told) I have to go and "learn to live with" my condition. I now feel I know more about my FSH than do GPs and to a point Neurologists and I can tell them what I think is going wrong next because they can't know

Inadequate support from social services and widespread lack of recognition about patients with disability was also mentioned.

> Even taking into account the DDA, I feel that people with any disability are not given a fair deal. I feel the old image of being disabled must mean "retarded" still stands. I do get very passionate in my feelings and write to the PM, TV, papers, all the time in an attempt to get those issues discussed more openly. Without these issues discussed, and from primary school, I do not feel that we will come very far.

7.4 Discussion

Postal survey responses supported and verified the interview data, with respondents reporting the influence of NMD across a broad spectrum of life areas. The data supports the diversity of impact experienced by patients and shows that the effects of NMD are of great importance to patients. These effects, already implied in the interview study, were explicitly represented in quantitative data of the survey. These data represented the degree to which NMD patients are personally affected by their condition.

The number of respondents expressing a positive impact of NMD across the domains was negligible except for the relationship items. The potential for illness to make

relationships closer and more satisfying has been documented in other studies (Padilla et al, 1990). Further study of this could lead to the development of techniques that might help patients undergoing stresses in close relationships.

The impact of NMD upon activities was not surprising considering the physical disability experienced by many patients. It is inevitable that the most immediate impact of muscle disease will be upon everyday activities such as washing, dressing, getting around and doing household tasks. The survey data goes further, representing the impact of NMD upon social and leisure activities. Even sedentary activities were affected as these often involved travelling to attend, or energy to concentrate upon. Employment was also affected considerably and was of great importance to those of working age. This was unsurprising, given that work tends to be the main activity in adult life, taking up the greatest amount of time, providing social contact and influencing patients' financial situation. It is also important to the identity of many people.

From patients' reports, the impact of NMD upon relationships was not quite as marked as its impact upon activities. This is likely to be because the effect of NMD upon relationships depends much more on the individual and their situation. From comments made in the survey, important factors mediating this impact include the social support available. The personality of their family members, and the patient's own disposition is likely also to mediate the impact of NMD upon relationships.

On the relationship items there were a number of interesting differences between the groups. For example, respondents in the congenital group reported less impact and

importance of disease impact upon their friendships, family and partner relationships than the other two groups. This may be because most patients with a congenital muscle condition have lived with the effects of their condition for a long time, in many cases from childhood. In such cases there is less likely to have been an abrupt or dramatic change in the patients' physical condition and the consequences associated with this. On the other hand, patients with an acquired condition are more likely to have experienced a substantial change in their physical condition. Such a change will doubtless put pressure on close relationships.

Indeed the data showed that there was considerable impact upon, and importance attached to patients' relationship with their spouse/partner. This is likely to be because this is frequently the closest and most confiding relationship. Patients with disability are also likely to depend more upon their partner/ spouse than upon anyone else, which may put a particular strain on this relationship. Indeed the QoL of caregivers has been found to be negatively affected in numerous studies (Hughes et al, 1999). This negative impact is undoubtedly linked to changes and adaptations that both patients and close family members have to make to their role in family relationships (Young and Kahana, 1989; Marks, 1998). Given the closeness of the relationship, the respondent's partner/ spouse is also likely to be the person most anxious/ worried about the patient's condition.

The comments made in the survey also revealed the difficulties experienced by some respondents in meeting potential partners or starting off relationships, an issue that was not explicitly mentioned in the interviews. It may be that these issues are more difficult to comment upon in a face to face situation compared to a confidential survey.

Patients' difficulties in explaining their muscle conditions were also frequently mentioned. Many respondents, particularly those with myositis found that people tended not to understand the nature of their condition and its effects. It may be that that this was more of a concern to patients due to the fact that myositis is less common than the likes of muscular dystrophy and is seldom heard about outside the medical profession.

The reported impact upon patients' perceptions and emotional well being was also high. High ratings were found on the perceived impact and importance of independence item and perception of the future item. These findings were understandable, given the progressive nature NMD and patients uncertainty about the future consequences of their condition. In particular, respondents commented upon their fears of becoming increasing dependent on other people.

Body image was also considerably affected, although less so in the ASP group. This might be due to the age of the respondents as these patients were older than the other groups. It may be that older people place less emphasis upon physical attributes. Their expectations of physical ability and appearance may also be lower than the expectations of younger people considering the greater number of older people who have physical disabilities that influence their appearance.

Many respondents commented upon coping strategies although they were not specifically questioned about this in the questionnaire. Coping strategies appeared to be more predominant in survey comments than they were in the interviews. This may be because patients felt they had to explicitly justify their responses to items in the

questionnaire, whereas in the interview they could describe their experience of NMD within the context of their own lives.

The issue of drug treatment was of particular importance to patients with an acquired, relapsing, remitting condition. Many reported medical conditions that had been brought on as a result of steroid treatment (e.g. cataracts, diabetes, high blood pressure). For many patients, the beneficial effects of drug treatment outweighed any negative effects. However, for a considerable proportion side effects were prevalent and held as 'extremely important'. It is therefore essential that QoL measures used to evaluate treatment in NMD capture negative treatment effects as well as those that are beneficial.

Qualitative analysis of the comments provided insight into patients' feelings about drug treatment, which were often very distressing. There were numerous comments about the weight gain brought on by steroid treatment and comments about effects upon mood and other medical complications. Such issues are of considerable importance to HRQL and must be addressed in QoL measures that are to be used with patients undergoing treatments that may have such harsh side effects.

7.4.1 Limitations and Considerations

The representativeness of the data may have been compromised by a number of factors.

1. Response rate.

The 47% response rate to the survey may have compromised how representative the data were (Mangione, 1995; Fowler, 1995; Fink, 1995).

There are a number of possible reasons for this somewhat low response rate to the survey. Most of the questionnaires (480, 89% of the total number sent out) were sent through patient support groups. This allowed larger numbers of patients to be reached but decreased control over the survey process. It would have been ideal to have conducted a more rigorous follow up to the initial mail-out of the survey as recommended in section 6.5.4, for example including postcard reminders as well as a second mail-out of the instrument. However this would have been difficult to implement given that most of the questionnaires were sent through support groups who would not have the time or resources to carry out such a labour intensive process. Possible reasons for non-response from some of the support group members are that:

- Some may not have had a firm diagnosis of NMD.
- Other members may have been relatives (e.g. parents) or friends of people diagnosed with NMD.
- The questionnaire was enclosed with separate newsletter mailings. This minimised postal costs, but may have compromised the salience of the survey to support group members and ultimately lessened the impetus to respond.
- Severely affected patients may have been unable to complete the form
- Some of the patients receiving the questionnaire may have been very mildly affected and therefore not considered their response to be pertinent.
- Patients with myositis whose condition was in remission may not have considered their response relevant to the survey

2. Representative sample?

It may be that those least and those most affected by NMD did not respond to the survey.

Members of patient support groups are unlikely to be representative of patients with NMD as a whole. For example:

- They may not have a firm diagnosis of NMD
- They may be more severely affected than other patients
- They may have a better support network.
- They may be better informed
- They may have higher expectations than patients who do not join support groups.

It is also possible that, as with all surveys, someone else, other than for whom the questionnaire was intended filled the questionnaire in. This may happen if the patient is too ill to complete the questionnaire, or if they have difficulty in holding or writing with a pen.

7.4.2 Implications of the survey

From the postal questionnaire data, it was clear that NMD has a considerable impact in all life domains. Patients also rated this impact to be important.

The wide-ranging picture of the NMD impact that an individualised measure, specific to patients with NMD would best capture disease impact. Such a measure would focus upon those aspects of life affected by muscle disease and considered to be important.

Looking at issues that are of particular relevance and importance to patients should make it easier to capture any improvement or deterioration in their well-being. This also takes account of any change in the patients' experience of disease and the importance attached to particular areas of life. Understanding such changes is likely to be beneficial in addressing patients' needs, not only in terms of treatment but also in the non-medical interventions and advice provided.

CHAPTER VIII

DESIGNING QOL QUESTIONNAIRES AND THE CONSTRUCTION OF THE INDIVIDUALISED NEUROMUSCULAR QUALITY OF LIFE QUESTIONNAIRE (INQOL)

Chapter 8: Designing QoL questionnaires and the construction of the Individualised Neuromuscular Quality of Life questionnaire (INQoL)

8.1 Introduction

Following the review of QoL literature and exploration of patients' experience of NMD, the new QoL questionnaire was constructed. The model that resulted from the literature review influenced the structure of the questionnaire. The interview and postal survey results determined its content.

Constructing QoL questionnaires involves selecting items and making decisions about the scaling, scoring and administration of the scale. These issues were examined in order to guide the construction of the Individualised Neuromuscular Disease Quality of Life Questionnaire (INQoL).

8.2 The Generation and Selection of Questionnaire Items

Item selection is important in ensuring that the questionnaire appears to measure what it is intended to measure (face validity) and that it is comprehensive in representing what it purports to measure (content validity).

The initial phase of questionnaire development increasingly involves qualitative methodologies to investigate the topic of interest and generate themes to form the content of the scale. QoL questionnaire items can be generated from multiple sources, including expert opinion, literature reviews, and the experiences of patients explored through qualitative interviews and focus groups (Streiner and Norman, 1995). Deriving QoL questionnaire items from patients' experiences is especially helpful in obtaining a representative view of patient QoL.

Item selection involves using either the psychometric method of factor analysis or the clinical impact method. These lead to the elimination of redundant or inappropriate items, reducing the scale to a feasible length but maintaining comprehensive coverage of the construct. Psychometric strategies such as factor analysis are based on mathematical techniques and are generally used to develop scales that measure a single characteristic or attribute (homogeneous scales) (Nunnally and Bernstein, 1994). Clinimetric strategies such as the clinical impact method rely largely on patients' and clinicians' judgements and tend to be used to develop measures comprising several characteristics or attributes (heterogeneous scales) (Feinstein, 1987).

8.2.1 Factor Analysis

Factor analysis can be used to determine which items and subscales belong to which underlying 'factor' of a construct such as QoL. It also determines the strength of the statistical relationship between factors and leads to the elimination of items that bear a weak relationship to the factor to which they should relate and items that contribute little to the overall questionnaire score. Items that load on (are correlated with) scales to which they should not relate may be measuring something other than the scale developer intended. Items are therefore not selected if they load on a factor to which they should not relate or if they load on more than one factor (Streiner and Norman, 1995).

The steps involved in item reduction that involve the use of factor analysis have been described in a number of papers (Hyland et al, 1991; Marks et al, 1992; Juniper et al, 1997; Marx et al, 1999).

- The skewness of data is examined and the items that are selected by the fewest number of respondents (less than 40%) are eliminated.
- Items that show low item-total correlation (less than 0.40), i.e. that contribute little to the aggregated score of the scale are left out.
- Items with the lowest item-total correlation from subgroups of items that appear to be measuring the same thing (i.e. that are highly correlated with each other) are also left out
- Items that load by less than 0.4 on the first factor (e.g. overall QoL) using principal component analysis (Hyland et al, 1991) are also removed.

8.2.1.1 Limitations of Factor Analysis

Factor analysis is suited to the analysis of scales that have only a few subscales, such as psychological scales (e.g. measures of depression or neuroticism). However its application is less appropriate in the examination of QoL scales as they commonly involve many subscales that contain just a few items each. This increases the likelihood that items from the different subscales will load on more than one of factor (e.g. social and emotional items might be closely related). The elimination of items is also likely to leave some individual subscales with very few scale items.

Factor analysis derives the factor structure of a data set using rotation techniques, which often results in many different factor solutions (Fayers and Machin, 1998). If too many or too few factors are entered into the model the resulting solutions may be difficult to interpret. Conversely, it is possible to derive plausible meanings from various different combinations of variables making it difficult to determine the most accurate factor model.

Conducting factor analysis also requires a particular sample size. If the sample size is very large there is a risk of extracting too many factors. On the other hand, a small sample size may result in insufficient information to enable more than one or two factors to be drawn out.

A further problem is presented by the categorical and asymmetrical nature of QoL data as the estimation techniques used alongside factor analysis assume continuous, normally distributed data. Taking this and the multidimensional nature of QoL data into consideration, it has been recommended that many hundreds of participants be used when using factor analysis to select questionnaire items (Fayers and Machin, 1998).

8.2.2 Impact Method

The clinical impact method (Juniper et al, 1997) selects items that patients identify most frequently and rate as most important (Feinstein 1987; Lacasse et al, 1999).

In studies comparing the item reduction methods of clinical impact and factor analysis (Juniper et al, 1997; Marx et al, 1999), the clinical impact method involved asking patients to rate the importance of each positively identified item on a five point scale (1=not important, to 5=extremely important). The frequency with which the item is identified and the importance attached to that item are combined into an impact score and items are then ranked according to their impact scores. The highest scoring items are ultimately selected for use in the questionnaire. This ensures the inclusion of only the most relevant and important items, resulting in a questionnaire that is more meaningful to patients and more responsive to change.

8.2.3 Comparison of clinical impact and factor analysis methods

Clinical impact and factor analysis both use a combination of empirical methods and intuitive judgement in the selection and grouping of questionnaire items. The clinical impact method involves the categorisation of items into domains according to clinical sensibility. Factor analysis is based largely on the correlation between items but involves subjective decisions, for example about the choice of 'cut point' for the item-total correlation determining the elimination of items and the decision about which factor structure is most appropriate.

Despite their differences, the methods of factor analysis and clinical impact have led to scales with largely similar items and domain structure (Marx et al, 1999; Juniper et al, 1997). Nonetheless, the clinical impact method may be favoured in the development of QoL scales given the problems cited with factor analysis and the value of incorporating the items of greatest importance to patients (Juniper et al, 1997). The clinical impact method also allows more flexibility in determining grouping of questionnaire items as it may make sense to group certain items under the same subscale (e.g. items on daily activities and leisure activities) regardless of the strength of correlation between them (Fayers and Machin, 1998).

8.2.4 Expert opinion

Following the application of either method of item reduction it is recommended that clinical judgement be used to ensure face validity (Marx et al, 1999). Experts are often asked to comment upon and criticise a rough draft of the scale, thereby influencing the final selection of questionnaire items. However, it is important for the panel of experts to represent a range of different disciplines in order to avoid bias in the final questionnaire. Furthermore, the use of expert opinion may not be appropriate

for questionnaires that are intended to focus upon patients' subjective experience. The face and content validity of QoL instruments should therefore be verified in a pilot study.

8.3 Questionnaire Administration

HRQL measures can be self-administered or administered by trained interviewers depending upon the time and resources available, patients involved in the study and the purpose of the investigation.

8.3.1 Interviewer-administered scales

Interviewer-administered questionnaires ensure compliance and decrease errors and missing items. However, the respondent may be less willing to acknowledge or admit problems in a face-to-face situation, which may lead to response bias. Social desirability is one of the biases commonly documented in questionnaire responses that is more likely when the questions are posed by an interviewer (Cook et al, 1993; Wynder, 1994). Responses are also influenced by the characteristics of the interviewer (e.g. gender, age or cultural background) particularly if the topic of the interview is of a sensitive or personal nature. For example, a survey of mental health in the USA found that male and female respondents were more likely to disclose information about their mental health (e.g. depression and substance abuse) to female interviewers than to male (Pollner, 1998). Interviewing style and the personality and experience of the interviewer may also influence response. This bias may be reduced through thorough training of interviewers. However, the time and resources involved in this training and in conducting the interviews limits the practicality of interviewer-administered questionnaires.

8.3.2 Self-administered questionnaires

The self-administered approach is considerably less resource intensive than interviewer-administered questionnaires. Forms tend to be easier to score and some can even be scanned by computer and used to provide rapid feedback to clinicians. Unfortunately, self-administered questionnaires involve more problems with misunderstood instructions, missing items, language and literacy barriers and a higher rate of noncompliance (Fletcher et al, 1992; Guyatt et al, 1993; Cella 1995). A compromise is to have instruments completed whilst under the supervision of an investigator who can check the respondents' comprehension of items and completion of forms (Cella, 1995; Guyatt et al, 1993). This is less time and resource intensive than interviewing each participant yet ensures higher compliance and more complete data.

8.3.3 Interactive questionnaires

On-line interactive questionnaires have also been introduced. These are similar to self-completed forms except that questions are presented on screen, sometimes even in touch-screen format (Velikova et al, 1999). Such questionnaires are quick and easy to administer and overcome the problem of missing items as the computer package can be programmed so ensure that each item is completed before moving onto the next. Respondents have also been found to prefer the more interactive questionnaires to paper versions (Velikova et al, 1999). Interactive questionnaires also have the potential to overcome literacy constraints if, for example, the scale is presented in a spoken format. This means that they may also be more appropriate for children than pen and paper measures.

Unfortunately limited resources mean that it will be a long time before interactive questionnaires are fully integrated into health care and research settings. Some patients may also have difficulty in using interactive methods if they are not familiar with them or if they are intimidated by the technology involved. Patients with physical disability may not be able to carry out the 'touch screen' or keyboard actions necessary to complete the assessment. However, these patients may have just as much trouble in completing a form with a pen or pencil.

8.4 Scoring QoL Questionnaires

8.4.1 Index versus Profiling Methods.

8.4.1.1 Indices

Despite widespread agreement about the multidimensionality of QoL instruments (e.g. Price, 1996; Bullinger et al, 1993), QoL is commonly represented as a single score in order to make the QoL data more amenable to statistics. However, the compression of such rich information into a single index prevents the identification of areas in which a change has taken place (Fletcher et al., 1992) and assumes that the individual subscales are equal in weight (Osoba, 1998). Aggregation of scores means that an overall QoL score could be identical for two patients despite completely different conditions and life profiles. For example, an agoraphobic, yet able-bodied individual may obtain the same overall score as a physically disabled patient with muscular dystrophy despite very different problems. Aggregated scores are also less sensitive to change and mask underlying changes on questionnaire subscales (Bullinger et al, 1993).

8.4.1.2 Single Item (Global) Indices.

Single item global indices (e.g. visual analogue scale or global improvement scales) are widely used to combat the statistical problems arising from the use of subscales and multiple scores derived from many different scales (Bech, 1993). The advantage of global scales is that they do not depend on the aggregation of unrelated subscales to produce an index score.

However, rating QoL on a single (i.e. global) scale is likely to lead to different interpretations about the meaning of QoL according to different respondents. This does not allow specific elements of quality of life to be pinpointed (Pearlman and Uhlmann, 1991) and presents a drawback in clinical practice where the identification of specific areas of concern can be useful in tailoring treatments and monitoring patients' progress.

8.4.1.3 Profiles

Instruments that result in a 'profile' of scores assess different components of a particular construct and represent the degree to which each area of life is influenced unfavourably by illness. The information provided can therefore be used to help make treatment decisions or to determine the changes underlying an improvement or deterioration in QoL.

The difficulties involved in the statistical analyses of profile measures make it important to make a priori statements about hypotheses and planned comparisons in order to minimise the likelihood of achieving significant results by chance. Statistical corrections such as the Bonferoni adjustment can also be used to adjust significance levels when carrying out multiple comparisons (Bullinger et al, 1993).

8.4.2 Weighting questionnaire items

Many scales use a weighting method to enhance the sensitivity of the questionnaire. This involves the allocation of greater weights to items that are more important to the overall end score. For example, if independence were found to be more important to patients than body image, the questionnaire item(s) relating to independence would be given a greater weighting than the body image items. This means that the item will have more of an influence upon the final QoL score.

A number of methods have been adopted for applying weights to questionnaire items.

8.4.2.1 Factor Analysis

Factor analysis has been used to assign weights to questionnaire items (e.g. NHP). However, the factors emerging from the analysis of one group may not be appropriate for another subgroup of patients (Fayers and Machin, 1998). This is because symptoms, side effects and QoL issues are likely to vary across different groups depending on factors including stage of disease, the patient's age and sex and the treatment adopted. Furthermore, in heterogeneous samples (e.g. different subgroups of patients) factor analysis may produce factors that represent group differences rather than distinct variables.

8.4.2.2 Regression methods

Regression weighting methods can also be employed to derive item weights (Bozzette et al, 1994; Diehr et al, 1995). The weights derived using this statistical method reflect the relative importance of individual subscales in predicting overall QoL (as measured by a criterion measure).

8.4.2.3 Importance ratings

Formal systems of deriving item weights do not guarantee that these weights are either clinically meaningful or meaningful to individual patients. A more novel and increasingly popular method is to weight the item using an importance rating provided by the respondent. Such methods are adopted in individualised questionnaires including the SEIQoL (O'Boyle et al, 1992; McGee et al, 1991) and the PGI (Ruta et al, 1994). The Disease Repercussion Profile (DRP; Carr, 1996), a measure of handicap, also adopts this method of weighting to determine the contribution of each item to scores on the questionnaire.

8.5 Construction of the INQoL

8.5.1 Deriving the content of the INQoL

8.5.1.1 Exploration of patient's experiences

Data from the qualitative interviews and the postal survey were used to construct the new questionnaire (Appendix E). The analysis of the interview transcripts led to the emergence of a number of life domains reported to be influenced by NMD (Chapter 4). Findings from the interview also supported the QoL model described in Chapter 1, which was used to construct the format and scoring scheme of the INQoL.

8.5.1.2 Item selection

The domains that emerged from the interview study were incorporated into the postal survey. The survey went on to be used to determine the prevalence of impact in these life areas and the importance of this impact to patients (see Chapter 7). This clinical impact data was used in selecting life domains for the questionnaire. The clinical

impact method was chosen over factor analysis as it was considered more appropriate to include domains of importance to patients rather than domains derived from a statistical model that may or may not accurately reflect NMD patients' concerns. The use of expert opinion was also considered inappropriate in selecting items that would specifically represent patients' experiences.

In order to ensure representative scores and thereby enhance questionnaire responsiveness, items were selected to cover a wide range of issues relevant to respondents at both the severe and mild ends of the spectrum. All the items in the questionnaire received high impact and importance ratings in the postal survey (see Chapter 7 for a more detailed discussion of the analyses). It was therefore unnecessary to rank items according to their impact ratings. Instead, all the domains except for one were incorporated into the final questionnaire. The 'perception of the future' domain was left out as it was considered to be particularly dependent upon personality and emotional state and therefore less likely to provide clinically meaningful data (i.e. demonstrate any significant response to intervention).

The 'emotions' section of the INQoL incorporates four items that were not specifically probed in the postal survey questionnaire. These include anxiety, depression, frustration and loss of confidence/self-esteem. These more specific emotions were frequently mentioned in the interviews and commonly volunteered in the comments section of the postal survey instrument. As these represented quite different types of emotion it was considered important to include them as individual items in the INQoL.

As both positive and negative effects of treatment were reported in the interviews and confirmed as important in the surveys, the final section of the questionnaire asks about patients' experienced and expected effects of treatment and the importance of these effects.

8.5.2 Structure of the INQoL and the contribution of the QoL model

The use of clear conceptual models in QoL assessment is important if the information gained from QoL measures is to be used in the application of clinical care (Wilson and Cleary, 1995; Carr et al, 2001). It was therefore important to have a theoretical basis on which to build the structure and content of the instrument.

The definition of QoL as the discrepancy between experience and the patients' expected or ideal state (Calman 1984; Cella and Tulsky, 1993) was used as the basis of the questionnaire. This conceptualisation is central to the QoL model outlined in Chapter 1. It is incorporated in questions asking patients to rate their current state in relation to their ideal state ('exactly as I would like to be') and the worst state they can imagine ('the worst I could possibly be') in the five life areas (questions 5bi, 6bi, &bi, iii, v, 8bi and 9bi).

QoL-as-process accounts (Leventhal and Cole, 1997) and the ICIDH-2 (Gray and Hendershot, 2000) are also important to the theoretical model of QoL outlined in Chapter 1. In line with these frameworks and findings of the interview study, the INQoL was designed to separate out the different stages of NMD impact. The effects of symptoms are separated from questions about activities, relationships, emotions, independence and physical appearance (body image) in order to determine whether changes in symptom scores relate to changes in life domain scores. Responses to the

life domain questions are maintained as separate domain scores to enable particular problems to be identified and monitored over time.

The questionnaire also partially encapsulates the response shift element of the proposed QoL model (p.38). As the items in the scale are fixed, rather than individualised, any change in the content of patients' ideals or goals would not be detected. However, changes in patients' internal standards and in the value they attach to the life domains of the scale should be discernible. Two of the three types of response shift may be captured by the questionnaire. Comparing perceived symptom severity with the impact upon satisfaction with life domains (questions 5bi, 6bi, 7bi, iii, v, 8bi and 9bi) should provide an idea of whether the patients 'internal standards' have changed independently from a change in perceived symptoms. This should help to determine whether any change in disease impact is due to a change in symptom severity or to the process of adaptation. Changes in the value of particular domains will be evident from changes in importance ratings.

8.5.2.1 Weighting the items.

Symptoms affect different individuals in different ways depending upon factors such as age, employment status, family situation and role, leisure activities, and psychological make-up. This makes it difficult to apply highly standardised questionnaires without considerable loss of information, given the differences in the contribution of particular issues to an individual's overall QoL.

The questionnaire therefore incorporates items that ask patients to rate the importance attached to difficulties caused by specific symptoms and the importance of NMD

impact upon satisfaction with each domain. Patients are also required to rate the importance of treatment effects.

The importance ratings will determine the particular relevance of domain scores to individual patients and should thereby enhance questionnaire responsiveness. The questionnaire score should therefore reflect whether the influence of NMD becomes more or less of an encumbrance upon QoL.

8.5.2.2 Individualised Questionnaire?

Considering the diverse effects of the different muscle conditions, the advantages of individualised questionnaires in eliciting QoL evaluations at a personalised level are very appealing. However, fully individualised questionnaires that elicit specific concerns are difficult to implement in either research or clinical settings, as they tend to require administration by a trained interviewer, which makes them both time- and resource- intensive. Completely individualised measures may not even capture all the important issues (Pearlman and Uhlmann, 1991) if these issues do not come to mind or if patients are uncomfortable volunteering them.

Individualised measures also tend to be conceptually more complicated than standardised questionnaires. They require respondents to have a reasonably high level of cognitive ability in order to complete the measures accurately. This excludes patients with lower levels of cognitive ability and/or educational level.

In light of these difficulties the INQoL uses the importance rating method adopted in many individualised questionnaires to achieve a more accurate representation of each individual's experience of NMD. It does not ask patients to generate their own

domains. This fixed domain structure requires less cognitive effort from the patient and should also make it easier to monitor any changes in particular areas of QoL on repeated administrations of the scale.

8.5.3 Scaling INQoL items

In the pilot study (section 8.6) GRS and Likert scales were tested along with differing numbers of response options (See Appendix D for example of GRS scales). This ensured that the scale represented both extremes of the spectrum and provided enough gradations to elicit a representative range of responses and thereby enhance responsiveness.

The provision of a representative scaling system and the incorporation of items that would encourage an even distribution of responses also aimed to enhance questionnaire reliability.

8.5.4 Scoring the INQoL

Responses to the questionnaire can be scored and presented as a profile. Scores reflecting symptom impact and the influence of NMD upon life domains are weighted using the patient's importance ratings.

In order to ensure good inter-rater reliability a scoring scheme was devised to provide clear and unambiguous instructions for scoring. A Microsoft Access scoring database has also been designed and is programmed to score questionnaires automatically according to an established formula.

8.5.4.1 Scoring the INQoL using an Access database package

The Access database system designed to automatically score the INQoL involves entering responses into a form displayed on the computer screen. The programme then scores the questionnaire and presents the profile of scores in a numerical format. Scores are presented as the percentage of maximum severity or impact, in order to facilitate statistical analyses. There are four scores that represent symptom impact, five that represent impact upon life domains and satisfaction with life domains and a composite QoL score representing the impact of NMD upon patients satisfaction with the five life domains. Treatment effects are represented by two scores. One of these represents the trade-off between the positive and negative effects of treatment and the other represents patients' expected treatment effect trade-off.

The Access programme has an algorithm incorporated to impute values for any missing items (Appendix F). A variety of imputation methods (Curran et al, 1998) ensure minimal loss of data from missing items. This system is designed to impute sensible values for those items left out by patients. For example, if the 'leisure activities' item is missed out (question 5a), the average value of the other items in this section is imputed. If fewer than half the items that make up each subscore are left out no score is calculated for that particular subdomain.

8.5.4.2 Scoring the INQoL by hand

The questionnaire can be scored by hand with the use of a scoring sheet (Appendix G) and if necessary, imputation methods for missing data. The scoring sheet describes the necessary calculations and provides boxes in which to mark down the subdomain scores and graphs in which to represent the profile of scores. This scoring method is, of necessity, somewhat complicated and use of the Access package is recommended.

However, scoring each form by hand is feasible, taking approximately 5 minutes per questionnaire.

8.6 Piloting the INQoL

The questionnaire was piloted in a total of 25 patients to ensure its acceptability. This involved pre-testing for appropriateness of layout, timeframe, response scaling, wording and content validity. During the pilot study the INQoL was revised twice after which it was re-piloted in different patients. 8 of the 25 patients completed the INQoL following final revisions.

8.6.1 Phase 1

8.6.1.1 Methods

The original version of the questionnaire (Appendix D) was piloted in 11 patients attending the outpatients clinic or for a separate research appointment. The time taken to complete the questionnaire was recorded and patients were interviewed once they had completed the scale. They were asked whether the questionnaire was easy to follow, and whether they liked the adopted wording, layout and scaling methods. Patients were also asked what they felt the questionnaire was about and whether it captured all the issues of importance with regard to the effects of their condition.

8.6.1.2 Results

The questionnaire took patients between 10-20 minutes to complete. A number of difficulties were encountered.

Patients expressed difficulty in quantifying the impact of their condition upon a graphic rating (GRS) scale, scaled from 0-10 and anchored at the extreme ends of the scale by verbal descriptors.

The timeframe 'over the last two weeks' was also problematic for a number of respondents and some found the organisation of questions confusing. They found it difficult to rate the impact of individual symptoms (e.g. pain, weakness)upon specific life domains and tended to answer these questions in terms of the overall impact of their condition.

8.6.2 Questionnaire revision and phase 2 of the pilot study

8.6.2.1 Methods

In order to address the difficulties encountered in the first phase of piloting a number of revisions were made to the scale.

The GRS was initially adopted as it is easier to score than a visual analogue scale yet avoids problems associated with patients' differing interpretations of Likert scale category labels. However, given problems with the GRS, 5- and 7-point Likert scales were pre-tested, following recommendations for optimal numbers of categories (Streiner and Norman, 1995). The scales were labelled with numbers as well as verbal descriptors in order to minimise problems with differing interpretations of the descriptive category labels. These verbal descriptors were chosen to reflect differing degrees of impact or importance and patients were asked to comment on their appropriateness.

The timeframe of the questions was also changed from 'over the last two weeks' to 'at the moment'. It was felt that this would overcome difficulties in recall of experiences

over a longer space of time but would avoid problems associated with asking patients how they feel 'today'. This is because the day on which patients attend to fill in a questionnaire is unlikely to be a typical day (i.e. hospital visit) and responses may also be more influenced by mood.

Confusion over question organisation was addressed by separating symptom questions from life domain questions and treatment questions. Therefore, rather than asking about the impact of individual symptoms upon each life domain, patients were asked about the severity of their symptoms, the difficulties caused by these and the importance of the difficulties caused by each symptom. Questions about the overall impact of NMD upon life domains, including independence and body image were incorporated into a separate section in the questionnaire. A separate section asking about perceived treatment effects and treatment expectations was also incorporated in order to circumvent difficulties with querying patients' expectations of treatment on individual symptoms. This also meant that patients not receiving treatment would be able to skip treatment questions more easily.

Using the same methodology the questionnaire was piloted in a further 6 patients

8.6.2.2 Results

The Likert scale proved to be much more acceptable to patients in this phase of piloting and the 7-point scale was favoured over the 5-point scale as respondents felt they could represent their position more accurately.

Phrasing the questions with the reference period, 'at the moment' was also more acceptable as patients interpreted this to refer to a recent and ongoing time period.

8.6.3 Phase 3

Following final revisions to response scales, the questionnaire was piloted in another 8 patients.

8.6.3.1 Results

Following final revisions, the questionnaire still took between 10 and 20 minutes to complete. Patients found the questionnaire to be also acceptable and to comprehensively cover relevant and important issues.

Patients judged the questionnaire to be about 'how you as a person are affected by your muscle condition', 'the way I feel' and 'how patients feel about their illness'.

8.7 Discussion

Drug treatments and other physical interventions used in NMD tend to be aimed, first and foremost at the amelioration of symptoms, such as pain, fatigue and weakness. However, the consequences of symptoms in areas of life such as personal relationships and employment are of great importance to patients. These effects can be addressed indirectly through the amelioration of symptoms or through some other action or intervention (e.g. change in job/ increased social support).

As existing measures do not look at how people evaluate and reach conclusions about the consequences of their condition (Hyland, 1992) it is difficult to interpret apparent changes in QoL. The INQoL was therefore designed to separate out the different stages of disease impact. Patients' perception of the severity of their symptoms and

the impact of these symptoms were separated from the effects of their condition upon particular areas of life.

Breaking down scores into symptom impact and the impact of NMD upon life domains means that the INQoL could help in tailoring interventions to individuals. It should also increase understanding about the effects of interventions and patients' evaluation of these effects upon symptoms and important areas of life.

Patients found the questionnaire to be easy to complete, comprehensive and representative of the effects of their muscle condition.

CHAPTER IX

THE CLINIMETRIC PROPERTIES OF QOL QUESTIONNAIRES

Chapter 9: The Clinimetric Properties of QoL Questionnaires

9.1 Evaluation of Health Status Measures

It is important for new QoL questionnaires to be thoroughly tested in order to determine their interpretability, reliability, validity, sensitivity to change and ease of use. This gives an indication of the degree of confidence we can have in the results of QoL studies. This is important if findings are to be used to implement changes in service provision and treatment.

The validation process is expensive and time-consuming and involves large numbers of participants (Reid, 1996; Fraser, 1993). It is therefore important to plan validation studies carefully and choose the most appropriate methods for determining the clinimetric attributes of QoL questionnaires.

9.2 Important Attributes of QoL Measures

9.2.1 Interpretability

It is important for the results obtained from QoL instruments to make sense. For example, the significance of a particular change in score will only become clear once the questionnaire has been used repeatedly. This is because a seemingly remarkable change may in reality reflect a minimal effect, whereas a small change in score in another instrument may signify a substantial effect. For example, a change of five points on the Beck Depression Inventory (BDI) (Beck et al, 1961) may signify a substantial change in depression (e.g. from moderate to clinical depression) whereas a change of five points on a pain VAS may not signify a remarkable change in an individual's level of pain.

9.2.2 Reliability

Reliability refers to the degree to which scores on a measure remain the same assuming that the attribute of interest has not changed. Therefore, if the attribute remains the same, a reliable instrument will yield the same score when administered on different occasions, in different conditions, or by different interviewers.

The reliability of an instrument also influences its validity, as measurement error compromises the ability of the instrument to accurately reflect the entity of interest. Despite this, good reliability does not guarantee the validity of the instrument. For example, individuals may achieve similar results on a repeated test of grip strength but this would clearly not be a good measure of their verbal ability.

Sources internal to the questionnaire influence its reliability. For example, if the wording is ambiguous or the if the scaling does not provide satisfactory response options it is less likely to elicit reliable responses. External factors, such as the person who administers the form or the setting in which the questionnaire is completed may also influence reliability. For example, even simple differences in instructions before a questionnaire is completed can result in differing responses from participants. These sources of variance can be difficult to identify and eliminate. However, various methods can be used to detect different types of reliability and help to identify sources of error.

9.2.2.1 Different types of reliability

9.2.1.1 Internal consistency

Assessing the reliability of scales that measure one-dimensional attributes such as depression often includes a test of their internal consistency. This is important as all

the items within such questionnaires should measure aspects of the same phenomenon. Good internal consistency is demonstrated by a high correlation between scale items (Nunally, 1978). However, scale developers usually aim for a moderate rather than a high correlation, as each item should measure a different aspect of the phenomenon.

One method commonly used to measure internal consistency is *split-half reliability.* This involves randomly dividing scale items into two sub-scales and correlating them. Higher correlation coefficients indicate greater internal consistency. *Cronbach's alpha* may also be used (Cronbach, 1951). This indicates whether internal consistency can be improved by excluding specific items from the scale.

Item-total correlation can also be used to determine internal consistency. This involves correlating each individual item score with the overall score, minus the contribution from that item (as this would artificially inflate the correlation) (Streiner and Norman, 1995). This helps to ensure that each questionnaire item captures an aspect of the phenomenon.

However, when it comes to assessing the reliability of multidimensional questionnaires it is not appropriate to test for internal consistency as dimensions within such scales should not relate closely to one another. For example, the dimensions within QoL questionnaires such as emotional and physical well-being may bear some relationship to each other but they should measure very distinct aspects of the construct. It is therefore inappropriate to test QoL measures for internal consistency.

9.2.2.1.2 Inter-rater reliability

If the measure is interviewer- administered or if responses provided are open to interpretation whilst scoring, the reliability of the instrument between interviewers or raters should be assessed. A clear scoring system that may not be influenced by the interpretation of different individuals should demonstrate good inter-rater reliability. As the INQoL is a patient-completed measure with a predetermined scoring system there was no need to look at inter-rater reliability.

9.2.2.1.3 Intrarater / Test-retest reliability

It is important that questionnaire scores remain stable when no change has occurred in the attribute of interest. This is determined by comparing the scores from the initial assessment with a repeat assessment conducted after a short space of time within which the attribute of interest is unlikely to have changed. Enough time should be left between assessments to minimise the chance that respondents will recollect their previous responses. If the instrument reproduces the result obtained in the previous assessment, the questionnaire is said to have intrarater or test-retest reliability.

The appearance of reliability may be compromised if there is any real change in the attribute of interest. This is particularly likely when measuring aspects of health in which change may occur over a relatively short period of time (e.g. fatigue). The test-retest reliability of multidimensional questionnaires may also be lower as they possess more dimensions in which a change could occur between test and retest.

9.2.2.2 Quantifying reliability

There are a number of ways to quantify reliability. The *Pearson product-moment correlation* has been used most widely. This measures the extent to which the

relationship between two variables can be explained by a regression line. However, this method is believed to be rather liberal in its estimation of reliability (Streiner and Norman, 1995). Like other measures of correlation, it measures the relationship between two scores, rather than their agreement. This means that scores obtained on the second administration of the scale could be consistently different from the initial score, and yet yield a high correlation coefficient due to a strong linear relationship. One further complication with this method arises if more than two sets of data have to be compared (e.g. if there are more than two interviewers). In this situation, several tests of correlation have to be calculated between the data sets and there is no way to combine their results into one score.

The *intraclass correlation coefficient (ICC)* circumvents this problem and allows a single coefficient to be calculated that represents the average correlation between the sets of scores (Streiner and Norman, 1995). Derived from analysis of variance (ANOVA), it is used to measure the relationship between any two measures in the same subject and unlike Pearson's correlation the ICC will yield a value of one only if all the observations in each subject are identical.

Cohen's kappa (Cohen, 1960) is used to measure agreement between assessments on binary scales, for example those that measure the presence or absence of an attribute. Kappa corrects for the agreement that would be expected to arise by chance by subtracting the proportion of agreement expected by chance from the observed amount of agreement. Agreement is then expressed as a proportion of the highest possible amount of agreement (i.e. a proportion of 1.00 minus the proportion of agreement expected by chance). *Weighted kappa* (Cohen, 1968) follows the same principles but it is adapted for use with categorical rather than binary data. This

method reflects the degree of agreement, with a difference of one category indicating less disagreement than a difference of two or more categories, an important consideration in assessing the reliability of a scale. This method is mathematically equivalent to the ICC, but is used when ratings are made on a categorical rather than an interval scale (Fleiss and Cohen, 1973).

Bland and Altman (Bland and Altman, 1986; Altman, 1991) present another method for measuring agreement that is useful in assessing test-retest reliability. This involves plotting the difference between the two observations against the mean of the pairs of observations and the standard deviation of the differences between these scores. This graph should also indicate the line of equality on which all points would lie if the two assessments elicited the same score. Finally, the limits of agreement are calculated, the upper and lower limits of which lie two standard deviations away from the mean of the difference between the scores. Greater agreement is reflected by a smaller discrepancy between the upper and the lower limits of agreement.

This method provides an informative summary of the data, and is enhanced by diagrams illustrating the mean difference in the scores (in scatterplots). It also shares an advantage with the ICC in that it measures agreement between scores rather than the strength of their relationship. However, it has an additional advantage of not depending upon the variation in the scores between subjects. Correlation coefficients are higher the greater the variation in scores between subjects which results in a misleading picture of reliability (Bland and Altman, 1986).

9.2.3 Validity

The validity of an instrument refers to the degree to which it measures what it is

intended to measure. It is easy to validate simple measures of observable physical entities such as height or weight. This is because an objective criterion of some known quantity such as length in centimetres can be taken and compared with the new measure (Bland and Altman, 2002).

It is more difficult to establish the validity of scales that attempt to measure more abstract concepts. There is no way to categorically confirm whether a score representing an artificial construct such as depression accurately captures the entity of interest. Quality of life is one such concept and QoL measures require thorough validation to determine whether their scores make sense and can be used to draw inferences.

9.2.3.1 Face Validity

Face validity refers to the extent to which an instrument appears to be measuring what it is intended to measure. This judgement is usually cast by experts but can also be established by pilot testing the measure in a small sample of the population of interest. This is particularly relevant in establishing the validity of QoL measures as patients are the indisputable experts when it comes to assessing the influence of illness on their own lives. Pilot testing questionnaires with patients is therefore useful in confirming their clinical relevance.

Face validity is unlikely to be an appropriate indicator of the usefulness of scales designed to assess socially undesirable attitudes or matters perceived by respondents to be of a personal nature (e.g. spending habits, sexual practices). In these cases instruments with high face validity may not glean representative results, as responses are less likely to be truthful. However, in the case of health status and QoL, face

validity is a useful measure of scale validity.

9.2.3.2 Content Validity

Content validity is a measure of whether the questionnaire comprehensively covers the attribute of interest (Nunnally, 1978). It is determined by looking at how well the instrument assesses all the domains of interest in terms of relevance and comprehensive coverage. Selecting items during instrument development is therefore crucial in the achievement of content validity. As with face validity, it is more likely to be achieved if QoL questionnaire items are developed from patients' experiences rather than from 'expert' opinion.

9.2.3.3 Criterion validity

The assessment of criterion validity involves comparing the results obtained using the new measure with those obtained using an established gold standard or criterion. For example, the validity of an evaluator's assessment of grip strength could be assessed against the gold standard of a calibrated grip strength dynamometer.

The two types of criterion validity are concurrent and predictive validity. Concurrent validity refers to degree of correspondence between two measures administered at the same time, such as Beck's Depression Inventory (Beck et al, 1961) and a clinical evaluation of depression. Predictive validity refers to the correlation between the measure and a value emerging at a later time point. An example of this would be preliminary tests of mathematical ability and GCSE maths exam results. Criterion validity also refers to the ability of a shorter version of an instrument (the test) to predict the results of the full-length index (the gold standard).

Well-established QoL measures (e.g. the SF36, Ware & Sherbourne, 1992; the SIP; Bergner et al, 1981) have been used in the past as standards with which to appraise other newly developed QoL questionnaires. However, their use may not always be appropriate given the lack of an agreed definition of QoL. Such measures are also likely to assess QoL from a different perspective than that of the new measure.

9.2.3.4 Construct Validity

Criterion validity is easy to establish if the attribute of interest is directly observable. However, when it comes to measuring more subjective aspects of health it becomes necessary to test the 'construct' that underlies the assessment.

Determining the construct validity of an instrument involves testing a series of hypotheses in order to see whether the new scale relates to other variables in the expected way (Bland and Altman, 2002). For example, does a measure of depression result in different scores for individuals who have recently experienced bereavement compared to those who have not? Does a measure of post-traumatic stress provide different scores in soldiers before and after real life combat?

The more frequently an instrument is used and the more situations in which it performs as expected, the greater the confidence can be instilled in its validity. However, it is important to revalidate measures when using them in a new setting or with a new population as the wording or questionnaire format may, when used in a different language or culture, emphasise issues that differ from the original aim of the scale.

There are two types of construct validity; convergent and discriminant validity.

Convergent validity refers to the relationship of an instrument to other measures of the same construct (e.g. the depression subscale of the Hospital Anxiety and Depression Scale and Beck's Depression Inventory) or to other related constructs (e.g. stress levels and blood pressure). A higher correlation between measures indicates better convergent construct validity. If a scale has discriminant validity it should not demonstrate strong relationships with instruments that claim to measure unrelated constructs (e.g. intelligence and pain).

9.2.4 Sensitivity/responsiveness

Responsiveness refers to the ability of an instrument to detect change in patients who have either improved or deteriorated but not in those who have remained the same (Hays and Hadhorn, 1992). It is therefore an important attribute of instruments used to measure outcome in clinical trials.

Ceiling and floor effects in questionnaires can be detrimental to their responsiveness. Measures that demonstrate a ceiling effect elicit a disproportionate number of scores at the upper end of the scale whereas floor effects refer to a disproportionate number of scores at the low end of the scale (Bindman et al, 1990). Scales that demonstrate these effects do not adequately represent the entity of interest, which means that they are unlikely to provide a representative measure of change. For example, in a QoL scale in which high scores indicate better QoL, a ceiling effect would leave little room to represent improvement in patients obtaining high scores. On the other hand, floor effects would result in a failure to detect deterioration in those with the worst scores.

9.2.4.1 Determining responsiveness

There are a number of methods that can be used to help determine the responsiveness

of a scale. The most simple methods involve comparing reports of change provided by patients or clinicians with measures of change in clinical status, intervening health events, and interventions of known or expected efficacy (Liang et al, 1985).

If responsiveness is measured over the course of an intervention it must be administered to two groups, one receiving treatment and the other a non-treatment control group. Scores can be compared using an unpaired t-test (Liang et al, 1985) or by conducting a repeated measures ANOVA and responsive measures should detect any change that occurs in the treatment group.

Responsiveness can also be evaluated by using the effect size statistic (Kazis et al, 1989). This is calculated by dividing the mean change in score by the standard deviation of the baseline score with variation in baseline score used as a reference against which to judge change. Effect size is most commonly used to measure change in clinical trials where the variation in scores obtained after baseline may have been influenced by the intervention. Its widespread use in clinical research and resultant familiarity means that it is better understood than other measures of responsiveness and therefore more readily adopted.

Despite this, it has been argued that as the effect size statistic does not include information about response variance it cannot be used to test the statistical significance of the response means (Liang et al, 1990). The standardised response mean (SRM) (Liang et al, 1990) was proposed to represent this variance. The only way the SRM differs from effect size is that the mean change in scores is divided by the standard deviation of these changes rather than by the standard deviation of the baseline scores. The responsiveness statistic (Guyatt et al, 1987) is similar to both

these methods but divides the mean change in score by the standard deviation of the scores of patients who do not change. The reasoning behind this is that the variation in those who remain stable provides a good estimate of background noise.

Receiver operator characteristic (ROC) curves are used as a gold standard to evaluate responsiveness. The area under the ROC curve estimates the probability that a score from one population chosen at random, will exceed a score from the other population (Pereira-Maxwell, 1998). Therefore, instruments that classify patients correctly as improved versus non-improved will have a larger area under the ROC curve.

Unfortunately the relative value of these methods is still not well understood and requires further investigation (Bolton and Wilkinson, 1998). The effect size statistic is therefore likely to remain the responsiveness statistic of choice given its familiarity to researchers and ease of use.

CHAPTER X

CLINIMETRIC EVALUATION OF THE INQOL: METHODS

Chapter 10

Clinimetric Evaluation of the INQoL: Methods

10.1 Introduction

In order to determine the clinimetric properties of the scale a number of studies were conducted.

- Construct validity was established by conducting hypothesis testing. Hypotheses were made about the relationships between subscales of the new QoL scale and a range of symptoms, functional disability, work-related factors, emotional well-being and so on.
- Reliability was assessed by test-retest reliability over a one-week period.
- A preliminary measure of responsiveness was obtained by administering the new questionnaire again after a period of three to six months, along with the other assessments administered at the first time point.
- In order to investigate the clinical utility of the INQoL a pilot study was conducted. The questionnaire was incorporated into patients' clinic appointment and an observational study was conducted to determine its influence on the consultation.
- The interpretability of the questionnaire was established over the course of the validation studies

10.2 Validity of the Questionnaire

10.2.1 Face Validity

Face validity tends to be based on expert opinion. However, as the aim was to develop a patient-centred measure it was considered more appropriate to determine

the face validity of the instrument based on respondents' feedback. This took place during the pilot study (section 8.6, p. 196) in which patients completed the INQoL and were interviewed about its acceptability and coverage of important issues.

10.2.2 Content Validity

The thorough exploration of patients' experiences in the qualitative interviews and postal survey helped to ensure the content validity of the scale. The pilot study (section 8.6) also verified content validity as patients were asked whether they felt the INQoL adequately covered the NMD-related QoL issues.

10.2.3 Criterion validity

As there is no accepted definition of QoL and no universally accepted criterion measure of QoL, criterion validity was felt to be an inappropriate measure of validity in this study. In its place, a series of hypotheses about NMD-related QoL were tested in order to determine the construct validity of the scale.

10.2.4 Construct validity.

In order to test the validity of the INQoL and its subscales, the hypothetical constructs underlying the scale were tested against a series of established measures. The following hypotheses were tested:

1. Patients with more functional disability will report a greater impact of NMD upon their activities and independence and will give higher ratings of symptom impact

2. Patients who perceive their NMD symptoms as more severe will report these symptoms to have a greater impact upon their lives.

3. Patients reporting difficulties with mobility and self-care tasks will report more difficulty in carrying out physical activities and greater dissatisfaction with their ability to carry out their activities.

4. Patients reporting difficulties with mobility and self-care tasks will report lower levels of independence and greater dissatisfaction with their degree of independence.

5. Patients who have less social support will:

   Ia. experience greater difficulties in social relationships.

   Ib. report a greater impact of NMD upon their quality of life.

   Patients reporting difficulties in social relationships will also:

   IIa. report more of an influence of NMD upon their relationships and greater dissatisfaction with these relationships.

6. Patients exhibiting high levels of depression and anxiety will report a greater impact of NMD upon their emotions and will be more dissatisfied with their emotional well-being.

7. Patients with a more negative body image will report more of an impact of NMD upon how they feel about their physical appearance.

8. Patients with low levels of QoL as measured by generic QoL scales will have more negative QoL as measured by the INQoL.

10.3 Instruments (Appendix H)

10.3.1 Generic QoL Questionnaires

Commonly used generic QoL measures were among the instruments used to test the construct validity of the INQoL.

These looked at the constructs hypothesised to be an important feature of HRQL in NMD, including the experience of symptoms and their psychological, social and functional well-being. The hypotheses related to predicted relationships between the subdomains of the validated questionnaires and those of the new scale.

10.3.1.1 Standardised questionnaires used in the validation

The **Nottingham Health Profile (NHP)** (Hunt et al, 1985; Hunt and McKenna, 1989) has 38 items that can be divided into 6 subscales; sleep, energy, emotional reactions, social isolation, physical mobility and pain. Responses are given on a binary, 'yes/no' scale and each item has an individual weight which, when added to the other items within the subscale, produce a combined maximum score of 100. Higher scores indicate greater impact or lower QoL within that domain. The NHP has been used in myositis patients, showing an impact of muscle disease across most of its subdomains, particularly energy, physical mobility and social isolation (Chung et al, 2001).

The **SF-36** (Ware and Sherbourne, 1992; Brazier et al, 1992) was selected as it is the most widely used generic measure of QoL and it has also been used in studies of myositis (Alexanderson et al, 1999). The 36 items of this self-completed measure cover 8 dimensions: physical functioning, social functioning, role limitations due to physical problems, role limitations due to emotional problems, mental health, energy/vitality, pain

and general health perception. Aggregated item scores are transformed into a score on a scale of 0 to 100. Higher scores indicate a better health state.

The SF-36 has been found to have good reliability and validity (Ware and Sherbourne, 1992; Brazier, 1992) and responsiveness (Garratt et al, 1993; Garratt et al, 1996; Harwood and Ebrahim, 2000).

The **Functional Limitations Profile** (Patrick and Peach, 1989) is the British adaptation of the Sickness Impact Profile (SIP) which has been used in numerous studies of QoL in muscular dystrophy (Ahlstrom et al, 1994; Ahlstrom and Gunnarsson, 1996; Ahlstrom and Sjöden, 1996; Pehrsson et al, 1994). It has 136 individually weighted items, which the respondent answers by ticking statements that apply and are due to his/her health. Twelve subscale scores, two dimension scores (physical and psychosocial) and the overall FLP score are obtained by adding together scores of items endorsed by the respondent, dividing the total by the maximum score possible and multiplying this by 100. This results in scores out of 100. Higher scores indicate greater limitation.

10.3.1.2 Individualised Questionnaires used in the validation

The **Patient Generated Index (PGI)** (Ruta et al, 1994) is an individualised QoL questionnaire based on Calman's definition of quality of life as "the extent to which our hopes and ambitions are matched by experience" (Calman, 1984). The final PGI score is designed to represent the extent to which reality falls short of expectation in the areas of life in which the patient would most value an improvement.

The version used in this study (www.dundee.ac.uk/epidemiology/PGI) asked respondents to think of the most important areas of life affected by their NMD and list up to five of these areas. These areas are rated according to how the patient would like

to be in each particular area. The patient then distributes 14 points over the these domains, spending more on those they would most like to improve. This weights the contribution of the individual domains, which are then combined to achieve a total PGI score out of 100 with higher scores indicating better QoL.

The PGI has not previously been used with NMD patients. However, both the INQoL and the PGI are based upon patients' satisfaction with life domains and use 'the way I would like to be' as a reference point against which to assess these domains. It was therefore hypothesised that PGI scores would correlate with the QoL score of the new questionnaire. A moderate rather than strong relationship between the scale scores was predicted as on the PGI, patients generate their own individual domains whereas the INQoL provides broad life domains. The domains are also weighted differently, the PGI involving spending points rather than rating importance.

10.3.2 Measurement of symptoms.

10.3.2.1 Muscle Weakness and Myotonia

Visual analogue scales (VASs) were adopted to measure the symptoms of muscle weakness and myotonia (muscle 'locking') as there are no existing measures that assess perceptions of these symptoms. VASs are widely used to measure patients' perceived symptom severity. They are simple, brief and easy to administer. The scales used consisted of a 100mm long line anchored at either end by the extremes of the symptom. For muscle weakness the labels were 'no weakness' and 'extreme weakness' and for muscle locking, 'no locking' and 'extreme locking'.

10.3.2.2 Pain

As VASs are commonly used to measure patients' experience of pain it was considered appropriate to use one here. The 100mm VAS was labelled at either end by the extremes of 'no pain'(0) and 'extreme pain'(100). The pain subscales of the SF36 and the NHP were also used to determine the validity of the pain question in the new questionnaire.

10.3.2.3 Fatigue

Chalder's fatigue scale (Chalder et al, 1993) was used to measure the degree of the patients' fatigue. This scale has eleven items that can be split into two subscales, physical fatigue (7 items) and mental fatigue (4 items). Items are scaled on a 4-point Likert scale ranging from 'less than usual' to 'much more than usual'. These are scored from 0-3 and result in scale totals out of 21 (physical fatigue) and 12 (mental fatigue), with higher scores indicating higher levels of fatigue.

The scale is intended to detect cases of fatigue in epidemiological studies and to detect change, for example, in controlled clinical trials. Despite its brevity it has been shown to be reliable and valid. A similar scale on which this scale was based has been used to measure fatigue in NMD patients (Wessley and Powell, 1989). This earlier scale demonstrated elevated physical fatigue scores but relatively low levels of mental fatigue in NMD patients.

The Energy/vitality items in SF-36 and NHP were also used to validate the fatigue dimension of the INQoL.

10.3.2.4 Measurement of Function

It was not feasible to measure patients' muscle strength, as this would have necessitated the involvement of either a trained physiotherapist or clinician. Therefore, in place of manual muscle strength testing, two functional measures were incorporated. These were a timed walk and a timed 'stands' test, simple tests that have been found to be very sensitive to change in the functional capacity of the lower limbs (Wade and Langton-Hewer, 1987). The 'stands' test required the participants to stand up from a straight-backed chair ten times (with the use of arms permitted). The timed walk involved the patient walking as fast as they could over a distance of 10 metres with the use of technical aids (e.g. walking stick) permitted. The results from these tests were compared to INQoL symptom scores as well as the activity and independence domain scores.

10.3.3 Quality of life domain measurement

10.3.3.1 Activities

The Physical Activities subscale of the SF-36 was used to validate the activities subscale along with the timed functional tests mentioned above.

10.3.3.2 Independence

The Barthel index (Barthel, 1956) and timed functional tests were used to validate the Independence dimension of the scale. The Barthel index was adopted as the most widely used and well-established measure of activities of daily living (ADL) (Patel et al, 2000; Wade, 1992). It has also been use to measure disability in children with Duchenne muscular dystrophy (Nair et al, 2001). It contains 10 items, which list statements that describe different levels of independence in a number of functional tasks (e.g. grooming, dressing, and bathing). Item scores are added up and result in a

score that ranges from 0, indicating the lowest level of independence to 20, which indicates the highest level of independence.

10.3.3.3 Social Relationships

It was predicted that the Social Relationships subscale of the FLP and the Social Role subscale of the SF-36 would be related to the Relationships subscale of the new questionnaire.

As there are no measures designed specifically to measure the impact of disease upon social relationships, the Social Support Questionnaire: six item version (SSQ6) (Sarason et al, 1987) was also adopted. This scale measures the amount of social support available to each respondent and their satisfaction with this support. This is represented in two subscale scores. The scale representing the number of people available to offer support (SSQ6-N) results in a score ranging from 0-9, and the scale that represents satisfaction with overall support (SSQ6-S) results in a score between 0-6, higher scores indicating greater social support. The scores obtained using the SSQ6 are similar to those found using the full length SSQ (Sarason et al, 1983) yet it is less burdensome to complete and easier to score (Sarason et al, 1987).

It was predicted that scores on the SSQ6 would correlate with scores on the Relationships subscale of the new questionnaire. It was also hypothesised that there would be a relationship between scores on the SSQ6 and the overall QoL score as social support has been found to mediate the impact of ill health on QoL (Newsom and Schulz, 1996; Lewis, Manne et al, 2001; Bennett et al, 2001).

10.3.3.4 Emotional Well-being

The Hospital Anxiety and Depression Scale (HAD Scale) (Zigmond and Snaith, 1983) was adopted to measure underlying psychological well-being. It is a brief, easy to administer instrument of patient anxiety and depression that is a reliable screening tool and valid measure of symptom severity. The scale has 14 items that consist of two subscales of 7 items. Each item has a 4-point scale that indicates the degree of severity. This is scored 0-3 with a maximum score of 21 on both scales.

Developed as a measure to screen for psychiatric symptoms in hospital patients, scores of 7 or less are interpreted as non-cases, 8-10 are uncertain cases and a score of 11 or more indicate a definite case, although the authors recommend that ultimate diagnosis should be based on clinical judgement.

NHP emotional reactions, SF-36 mental health and FLP emotion subscales were also predicted to be related to the emotions subscale of the INQoL.

10.3.3.5 Body Image

Most measures of body image have been developed for the assessment of body image in people with eating disorders (Ben-Tovim and Walker, 1991). However, the impact of physical conditions upon body image has received increasing attention, for example in breast cancer (Mock, 1993) and deforming physical conditions such as rheumatoid arthritis (Gutweniger et al, 1999).

The Arthritis Body Experience Scale (ABES) (Williams and Barlow, 1998) was developed to measure the body image of patients with rheumatoid arthritis. It has nine item which are divided into two dimensions; Body Totality (5 items) and Body Self-

consciousness (4 items), both of which have high internal consistency (personal communication with B. Williams, 2000). This scale was considered relevant and appropriate to NMD patients. It was therefore adopted and only required small changes to three items. This involved substituting the word 'arthritis' for 'muscle disease'. Items are scaled from 1-10 and subscale scores are derived by adding the scores of each item, to achieve a score between 0 and 50 for Body Totality, and 0 and 40 for Body Self-consciousness. Higher scores indicate a more positive body image.

Table 10.1: Summary table of the predicted relationships between the scales

SUBSCALES OF THE QUESTIONNAIRE	PREDICTED CORRELATION WITH:
Weakness score	Weakness VAS
Pain score	Pain VAS Pain subscales of the SF-36 and the NHP.
Fatigue Score	Energy subscale of the NHP Fatigue scale (Chalder et al, 1993) - Total score - Physical fatigue score
Muscle 'locking' score	Muscle 'locking' VAS
Body image (physical appearance) score	Adapted Arthritis Body Experience Scale (ABES): Body totality score Body self-consciousness score
Activities score	SF-36: Physical activities Timed functional tests
Independence score	Barthel Index Timed functional tests
Social relationships score	Social Support Questionnaire (SSQ6) SF-36: Social Functioning FLP: Social Interaction NHP: Social Isolation
Emotional impact score	HAD Scale FLP: Emotion NHP: Emotional reactions SF-36: Mental Health
NMD-related QoL score	PGI FLP total score SSQ6-S & SSQ6-N

10.4 Recruitment

Patients from King's College Hospital clinics were sent a letter of invitation to take part in the study along with a patient information sheet and consent form. Those who wished to take part completed the consent form and returned it in a prepaid envelope. These patients were contacted by telephone to arrange their study appointment.

Patients were also recruited through patient support groups; the Myositis Support Group (MSG) and the Muscular Dystrophy Campaign. These patients were invited either by letter or during the annual general meeting of the MSG. Those recruited at the meeting were given booklets of questionnaires to complete at home and return by post. Those who accepted the invitation either attended King's College Hospital for a study appointment or elected to have the questionnaires sent to them by post for completion at home.

10.5 Study Appointments

The appointments took between half an hour and one hour and a half. Patients were allocated to a specific group and asked to complete the subset of questionnaires and assessments designated to that group (either list A or list B. See table 10.2). This was done to reduce the burden of completing a large number of questionnaires and thereby maximise the representativeness of the data gathered.

Questionnaires were administered in a random order to reduce the influence that particular questionnaires might have on responses to subsequent questionnaires. Questionnaires were randomised by assigning a number to each questionnaire and arranging them in packs according to number sequences listed in random number tables.

Table 10.2: Questionnaires and Assessments used in the Validation study (Appendix G for copies of the forms)

LIST A	LIST B
INQoL The MOS SF-36 (Ware and Sherbourne, 1992) (Brazier et al, 1992) The Patient Generated Index (PGI) (Ruta et al, 1994) The Fatigue Questionnaire (Chalder et al, 1993) A subjective pain visual analogue scale (VAS) A subjective weakness VAS A subjective myotonia / 'locking' VAS The Arthritis Body Image Scale (ABES) (Williams and Barlow, 1998)- Adapted for Neuromuscular Disease (ABES-r) Barthel Index (Mahoney et al, 1958) Functional assessment - Timed walk (10metres) - Timed stands test (10 stands)	INQoL The Functional Limitations Profile (FLP) (Patrick and Peach, 1989) The Nottingham Health Profile (NHP) (Hunt et al, 1985) Social support questionnaire- Brief version (SSQ6) (Sarason et al, 1987) Hospital Anxiety and Depression Scale (HADS) (Zigmond and Snaith, 1983)

10.6 Reliability Study

As the scale was designed to be able to detect change over time it was important to determine its test-retest reliability. A one-week period was considered short enough to minimise the chance that change would occur in any of the dimensions and long enough to reduce the chance of patients recollecting their previous responses. Patients were also requested to complete a 5 -point subjective change question on their second completion of the scale in order to determine whether they had experienced any change in their condition since the previous appointment.

A number of respondents elected to complete the questionnaire at home and post it back rather than return for a second appointment one-week later. This was due to the difficulties involved in travelling to attend the study appointment.

10.7 Responsiveness study

Patients taking part in the responsiveness study were asked to return to complete the same set of questionnaires (table 10.2, list A) after a period of 3-6 months. Symptom scales and timed functional tests were administered at both time points to demonstrate any physical or functional changes over this period of time. As the SF-36 was also administered at both time points in order to detect change in individual life domains. As the PGI is based on a similar theoretical premise to the INQoL, patients were also asked to provide a rating on a 5-point scale to indicate whether or not they had experienced a change in their condition since their first study appointment. This rating was used in order to determine whether a perceived change in their condition corresponded to changes on subscales of the INQoL.

10.8 Statistics

10.8.1 Reliability

As the INQoL is multidimensional with only a few items within the individual subscales it was inappropriate to test its internal consistency. The assessment of reliability therefore concentrated on the INQoL's test-retest (intrarater) reliability.

Correlation methods were considered inappropriate in assessing reliability as they tend to result in a spuriously high appearance of reliability given the high correlation coefficients that are bound result from scores on the same scale. It was deemed more appropriate to obtain a measure of agreement. For this reason, Bland and Altman's

(1986) method for calculating limits of agreement was adopted to assess the size of the differences between the first and second set of scores. This method also has the advantage of presenting a meaningful visual display of scatterplots that indicate agreement between individual's scores at test and retest. Confidence interval analysis determined upper and lower levels of agreement.

10.8.2 Validity

Hypothesis testing was conducted using Spearman's correlations. The use of a priori hypotheses ensured a robust validation process (Jaeschke and Guyatt, 1990). This meant that instead of testing all possible combinations, specific scores were correlated in order to minimise any false relationships likely to arise by chance.

10.8.3 Responsiveness

The responsiveness of the questionnaire was ascertained using effect sizes. An effect size of 0.8 or more is considered to reflect a large change, 0.5-0.8 reflects a moderate change and 0.2-0.5 reflects a small change. The effect sizes of all the scales used in the study were calculated in order to see if changes in score could be related across the various measures and particularly to changes in the new scale.

CHAPTER XI

RESULTS AND DISCUSSION OF THE VALIDATION STUDY

Chapter 11: Results and discussion of the validation study

11.1 Results

11.1.1 Patients

Altogether 95 people completed the questionnaire on at least one occasion during the study. The male: female ratio of the entire sample was 1: 2.2 and the mean age was 55 (SD 17.08, min-max 19-97). Patients were made up of three subgroups; 35 patients with a CSP NMD, 50 patients with an ARR NMD and 10 with an ASP NMD. Table 11.1 presents a summary of the number of patients who took part in each part of study.

Table 11.1: Number of patients taking part in each study

	Validity 1 Allocated measures from subset A	**Validity 2** Allocated measures from subset B	**Test-retest**	**Responsiveness**
Validity 1	54	26	35 †	25
Validity 2	-	60	27	5
Test retest	-	-	40	13
Responsive-ness	-	-	-	25

The cells indicate the numbers taking part in one part of the validation study who also completed another part (e.g. † 35 patients completing the questionnaires from list A also took part in the test-retest reliability study).

Table 11.2 provides a description of the patients in each of the sub-samples who took part in the validation studies. In the test-retest studies most of the respondents who completed the scale for the first time during a visit to the hospital elected to complete the scale at home on retest due to long distance involved in travelling to attend the research visit. All those who completed the scale for the first time at home also completed it at home on retest, returning their forms by post. All but three of the respondents taking part in the responsiveness study completed the scale in clinic on

both occasions. These three patients were unable to return for their final appointment and therefore completed the forms at home.

Table 11.2: Sample characteristics of patients participating in each study

Patient subgroups
CSP= congenital, slowly progressive neuromuscular disease
ARR= acquired, relapsing remitting neuromuscular disease
ASP= acquired, slowly progressive neuromuscular disease

Study (N= patients)	Patients	% of sample female	Mean age	Number of patients by subgroup
Validity 1 (N=54)	32* 22†	57%	52 SD= 13.86, min-max 22-86	29 CSP 19 ARR 6 ASP
Validity 2 (N=60)	15* 45†	81%	57 SD= 19 min-max 18-97	14 CSP 44 ARR 7 ASP
Test retest (N=40)	time1= 22*, 18† time 2= 5*, 35†	55%	53 SD= 16 min-max 22-86	19 CSP 14 ARR 7 ASP
Responsive-ness (N=25)	time 1= 25* time 2= 22*, 3†	48%	51 SD= 13.4 min-max 28-77	19 CSP 4 ARR 2 ASP
Total sample (N=95)	47* 67† 19 completed validity studies 1 & 2	70%	55 SD= 17.08 min-max 18-97	35 CSP 50 ARR 10 ASP

* Patients completing scales in clinic

† Patients completing questionnaires at home and returning them by post

The test-retest reliability and responsiveness studies are split into time 1 and time 2 indicating how many completed the scales in clinic and how many at home on first and repeated administrations.

11.1.2 Response profile of the patient sample

A description of the mean scores on individual subscales of the INQoL is displayed in table 11.3. The data provided is based on patients' first completion of the scale.

Table 11.3: Mean scores on the INQoL from the first completion of the 95 patients taking part in the validation study.

Higher scores indicate greater negative impact

Questionnaire dimension	Possible range of scores	Mean (SD)	Range (min-max)
Weakness	0-100	54.7 (27.68)	0-100
Muscle 'locking'	0-100	16.5 (26.66)	0-90
Pain	0-100	28.2 (29.99)	0-100
Fatigue	0-100	49.8 (32.81)	0-100
Symptoms	0-100	38.1 (22.38)	0-100
Activities	0-100	49.7 (29.18)	5-95
Independence	0-100	38.6 (30.40)	0-100
Relationships	0-100	22.9 (20.08)	0-95
Emotions	0-100	40.7 (27.32)	0-100
Body image	0-100	43.8 (29.57)	0-100
Perceived treatment effects	-100 to +100	16.9 (39.88)	-75 to100
Expected treatment effects	-100 to +100	39.88 (37.7)	-100 to 100
NMD-related QoL	0-100	44.2 (24.51)	0-95

Negative scores for perceived or expected treatment effects indicate that patients' perceived or expected negative effects outweighed the positive effects. Positive scores for treatment effects indicate that perceived or expected positive effects outweighed the negative effects.

Figures 11.1-11.12 show the number of respondents scoring within each ten-point interval on the scales of the questionnaire (0-100). The wide distribution of scores indicates that INQoL dimensions capture a wide range of disease impact. Roughly equal numbers of patients obtained scores ranging from the minimum (0) to the maximum (100) score possible for Weakness (fig 11.1) and Fatigue (figure 11.4). A large number of respondents did not report muscle 'locking' (figure 11.2) and a third of patients did not report pain (figure 11.3). This accounts for the skewed distribution of scores on these dimensions. Of those patients who did report these symptoms, scores obtained were evenly distributed across the range of possible scores.

Figure 11.1: Distribution of weakness scores

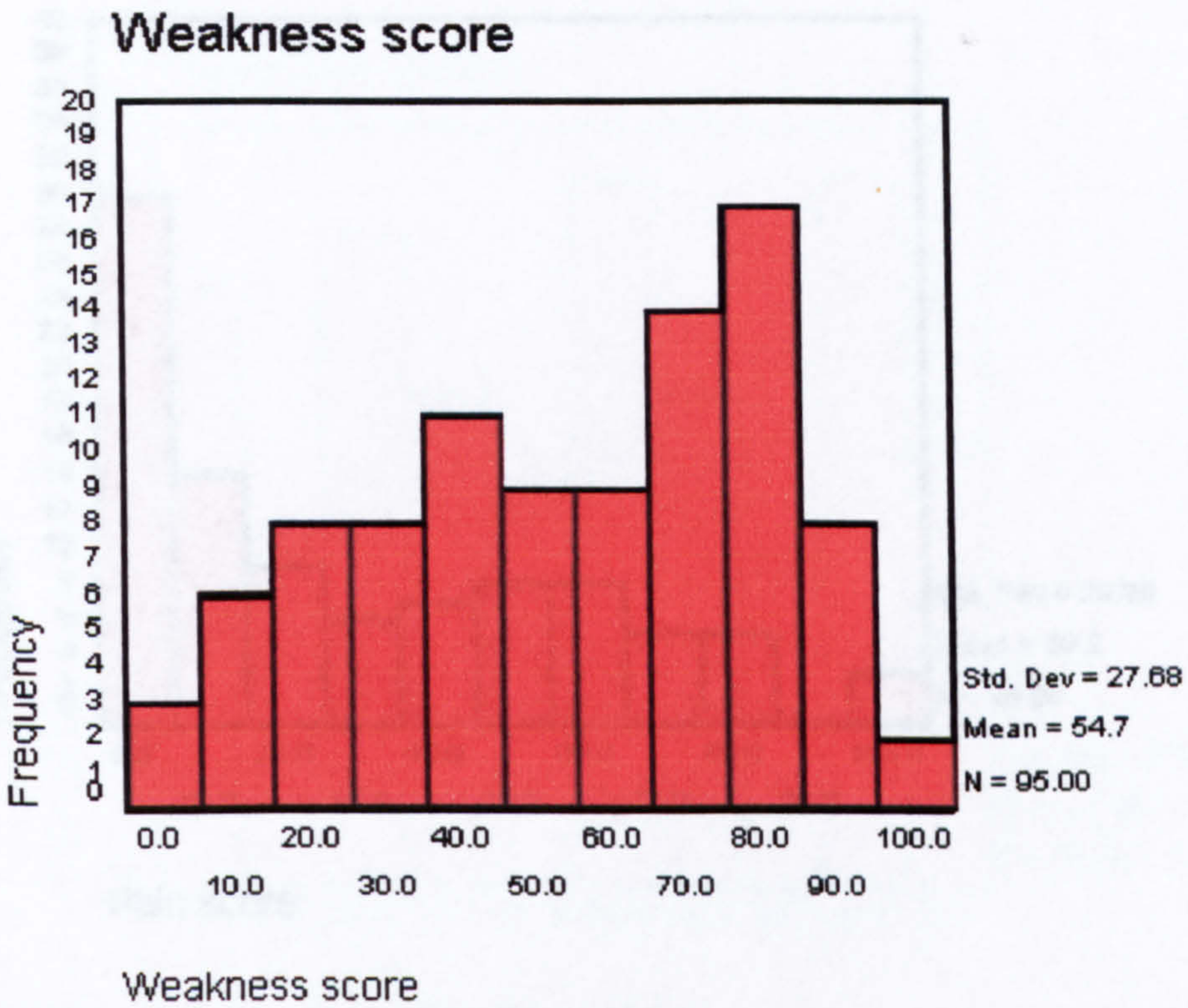


Figure 11.2: Distribution of 'Locking' scores

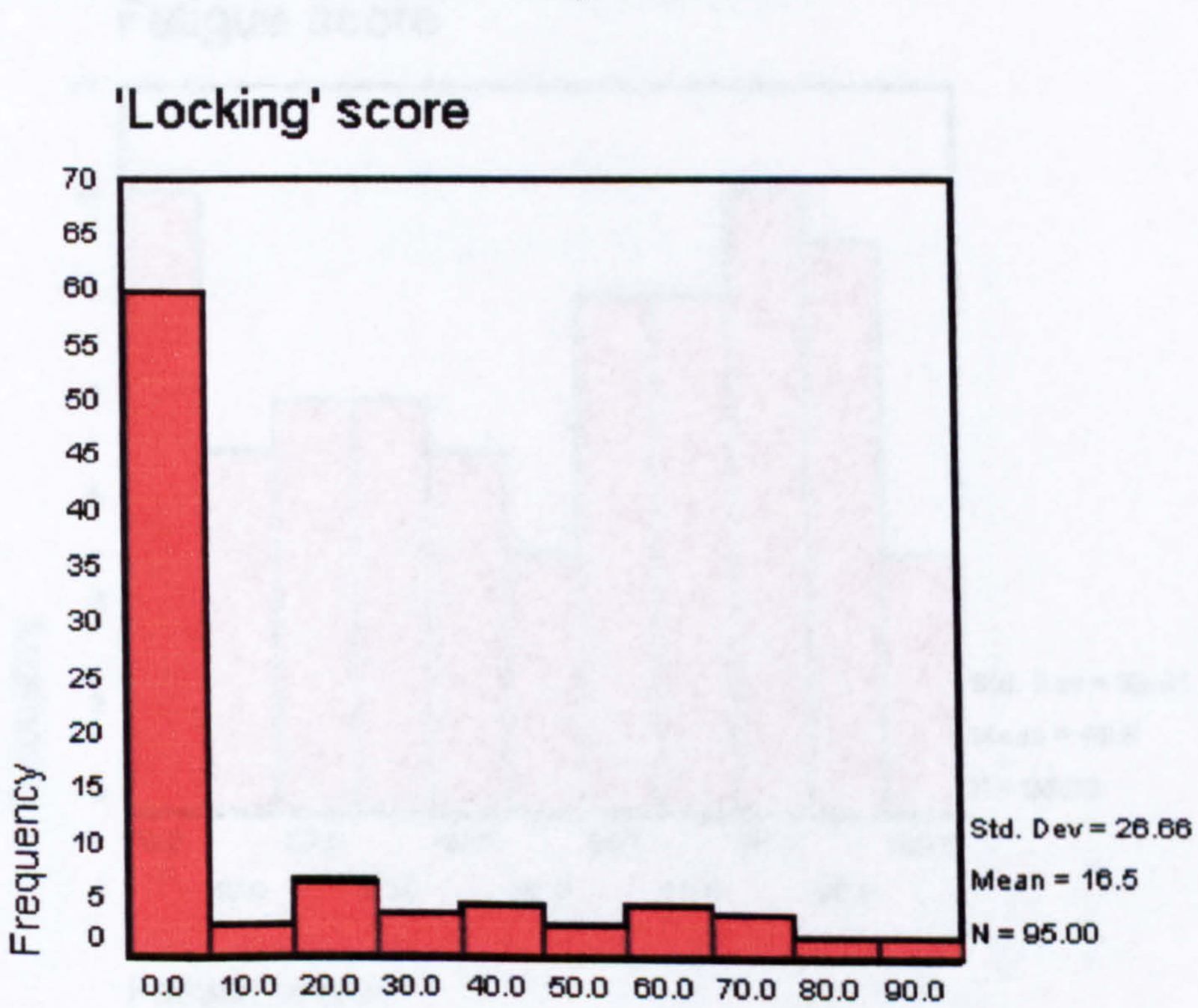


Figure 11.3: Distribution of Pain scores

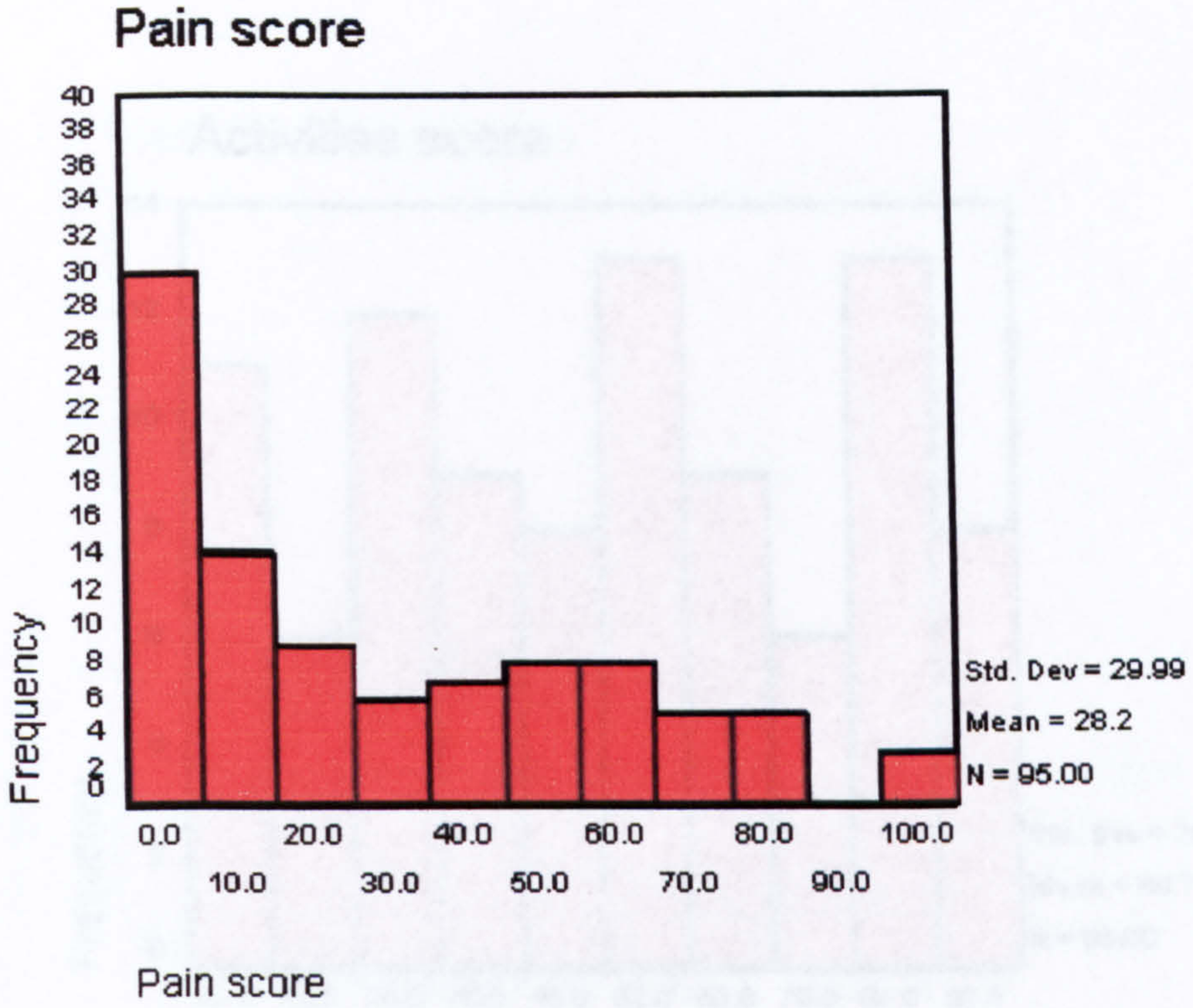


Figure 11.4: Distribution of Fatigue scores

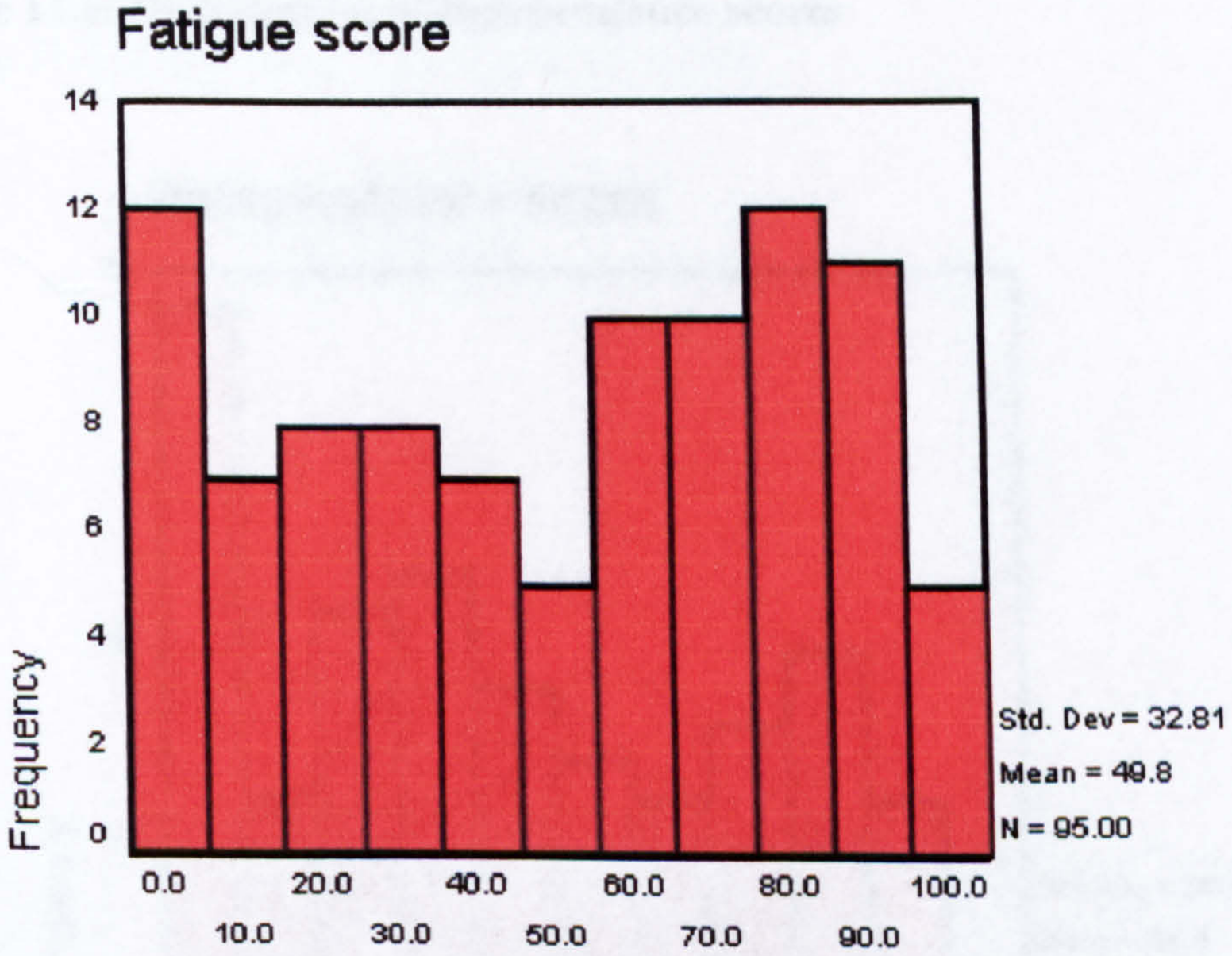


Figure 11.5: Distribution of Activities scores

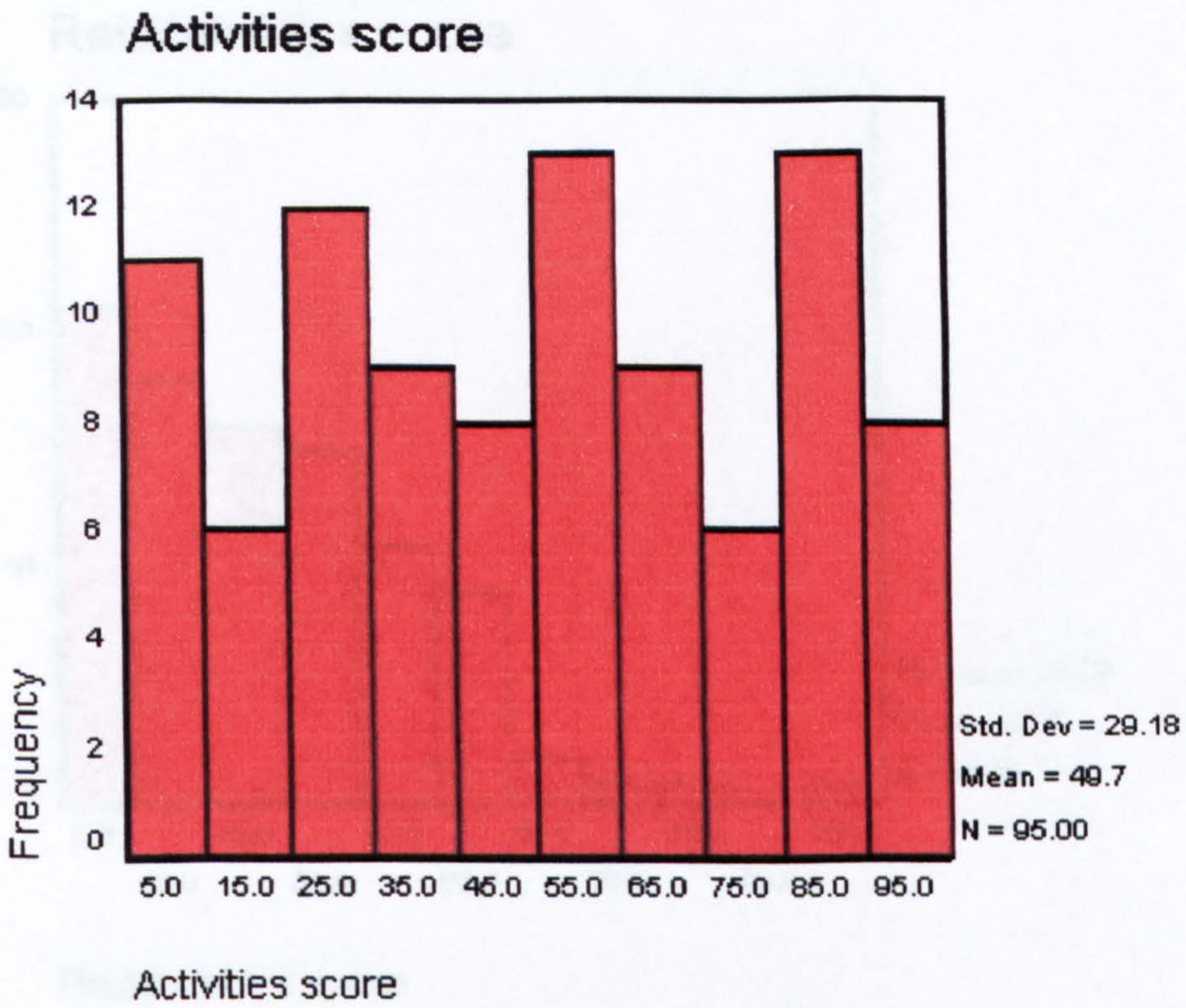


Figure 11.6: Distribution of Independence scores

Figure 11.7: Distribution of Relationships scores

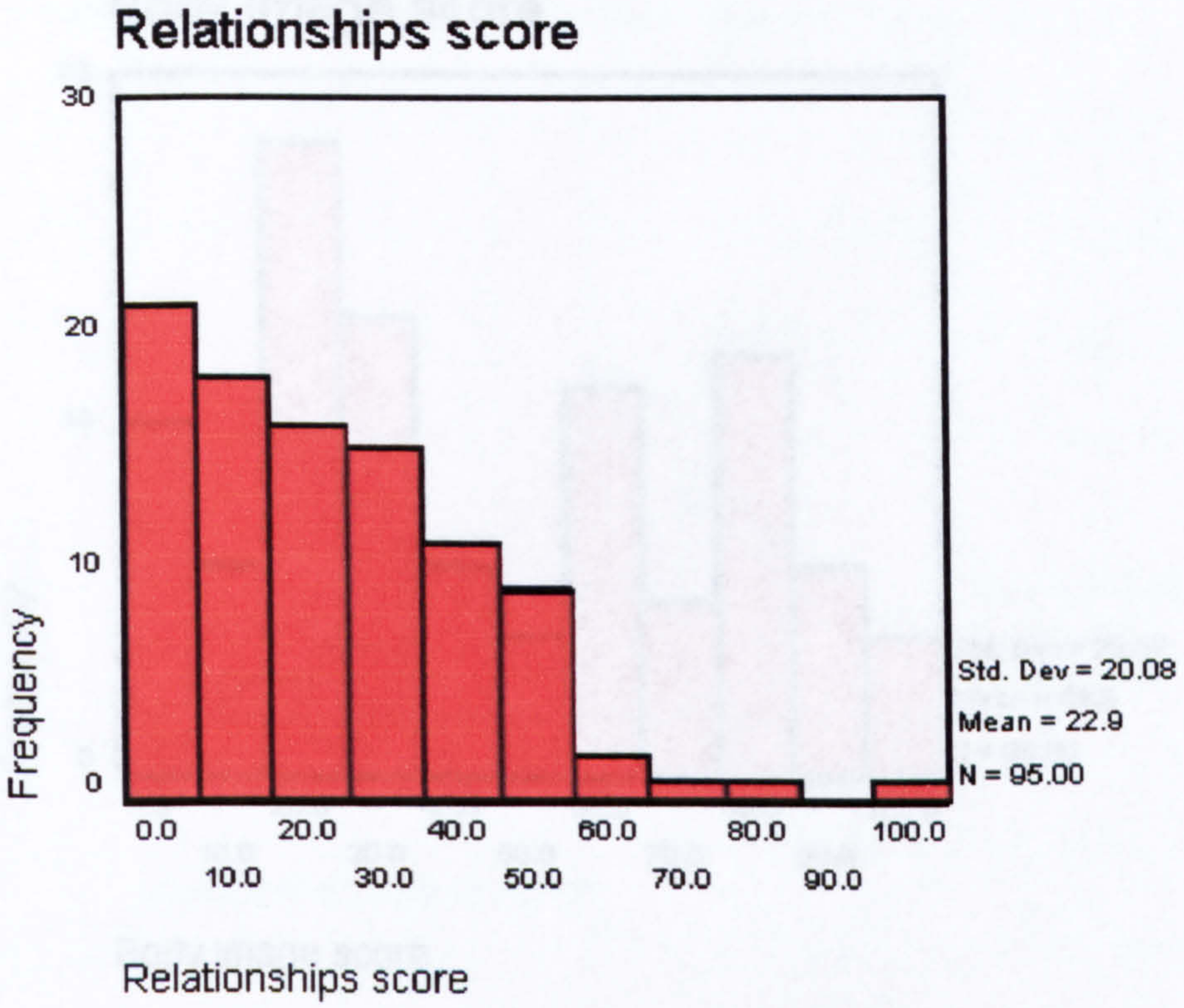


Figure 11.8: Distribution of Emotions scores

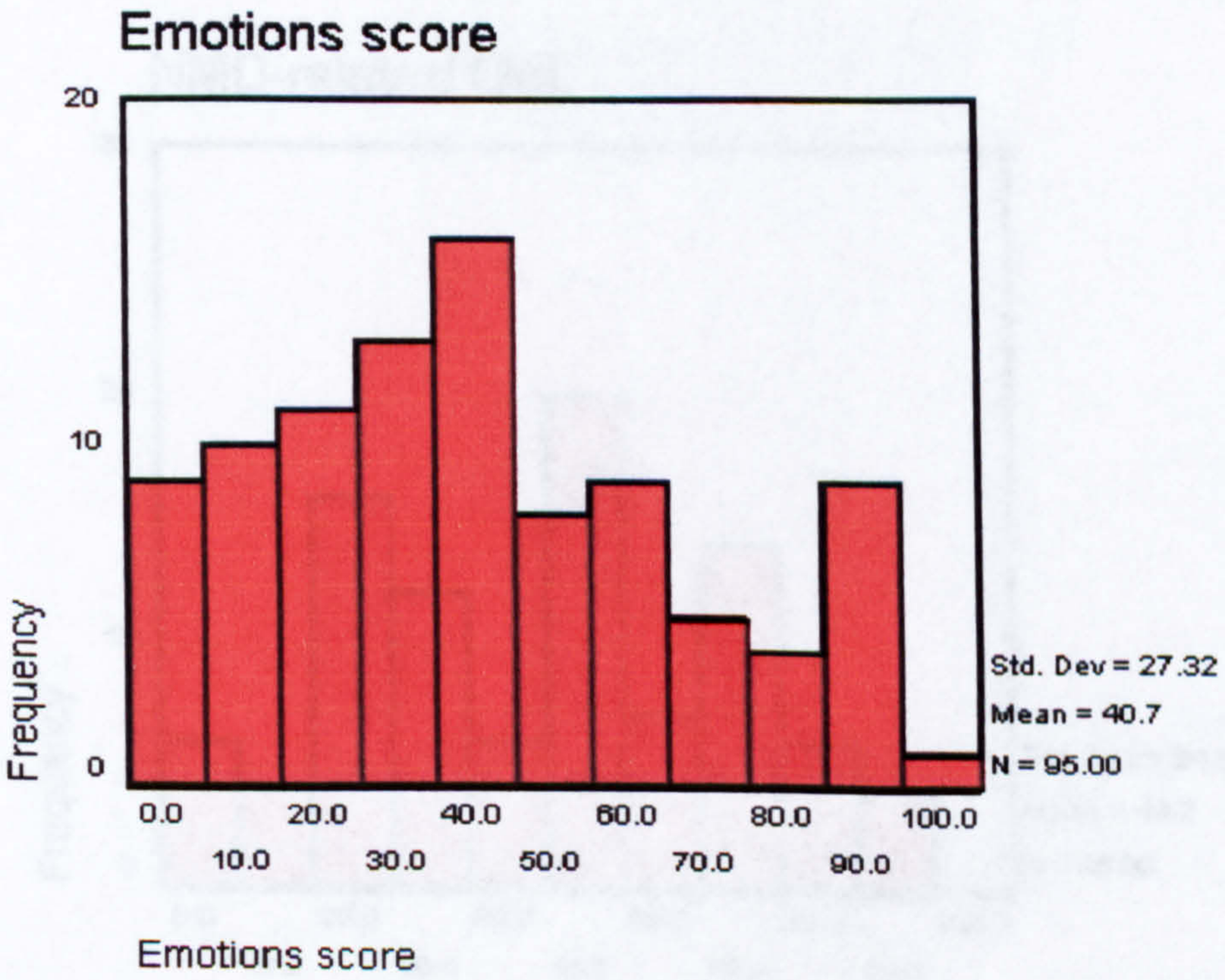


Figure 11.9: Distribution of Body Image scores

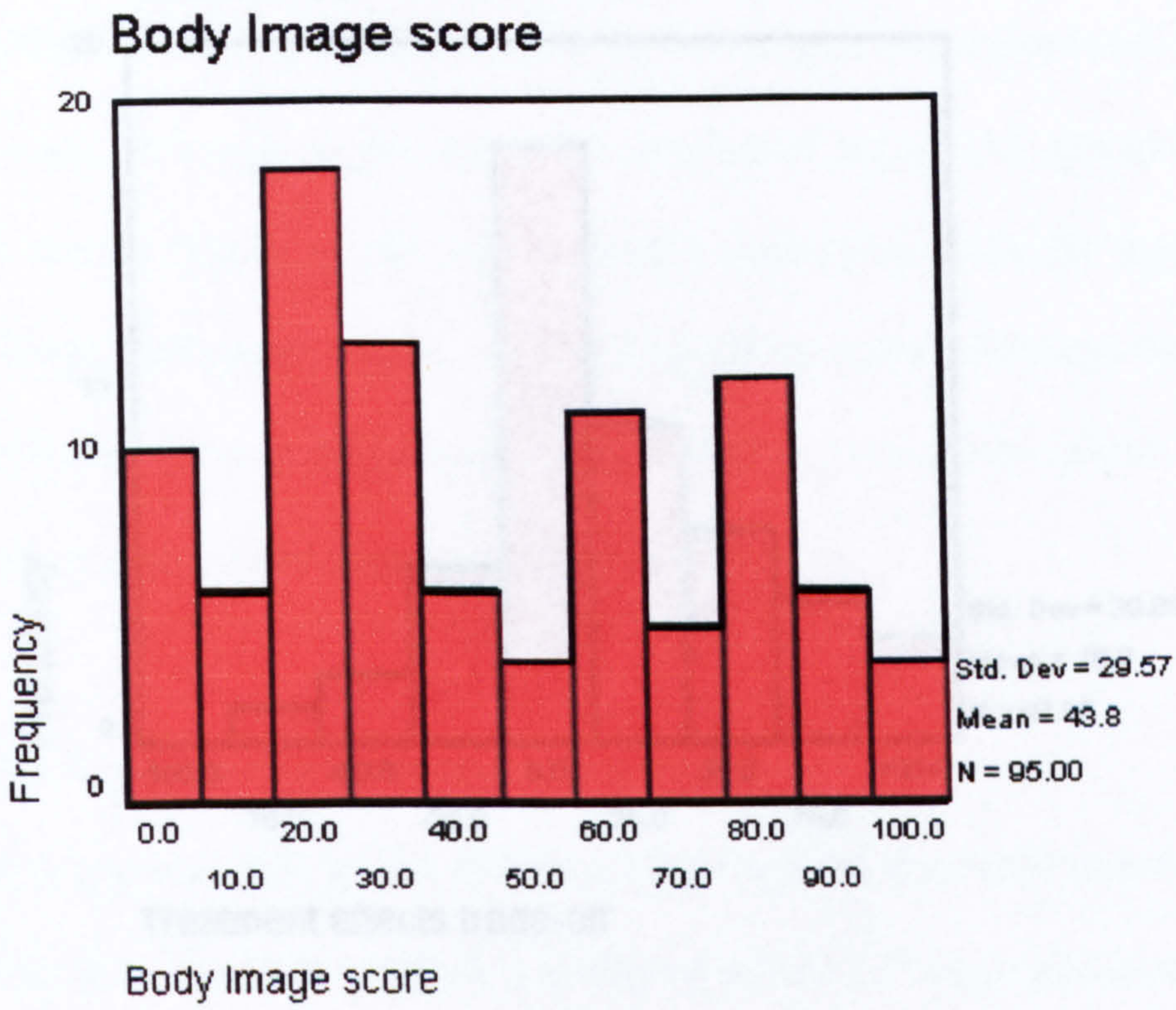


Figure 11.10: Distribution of NMD-related QoL scores

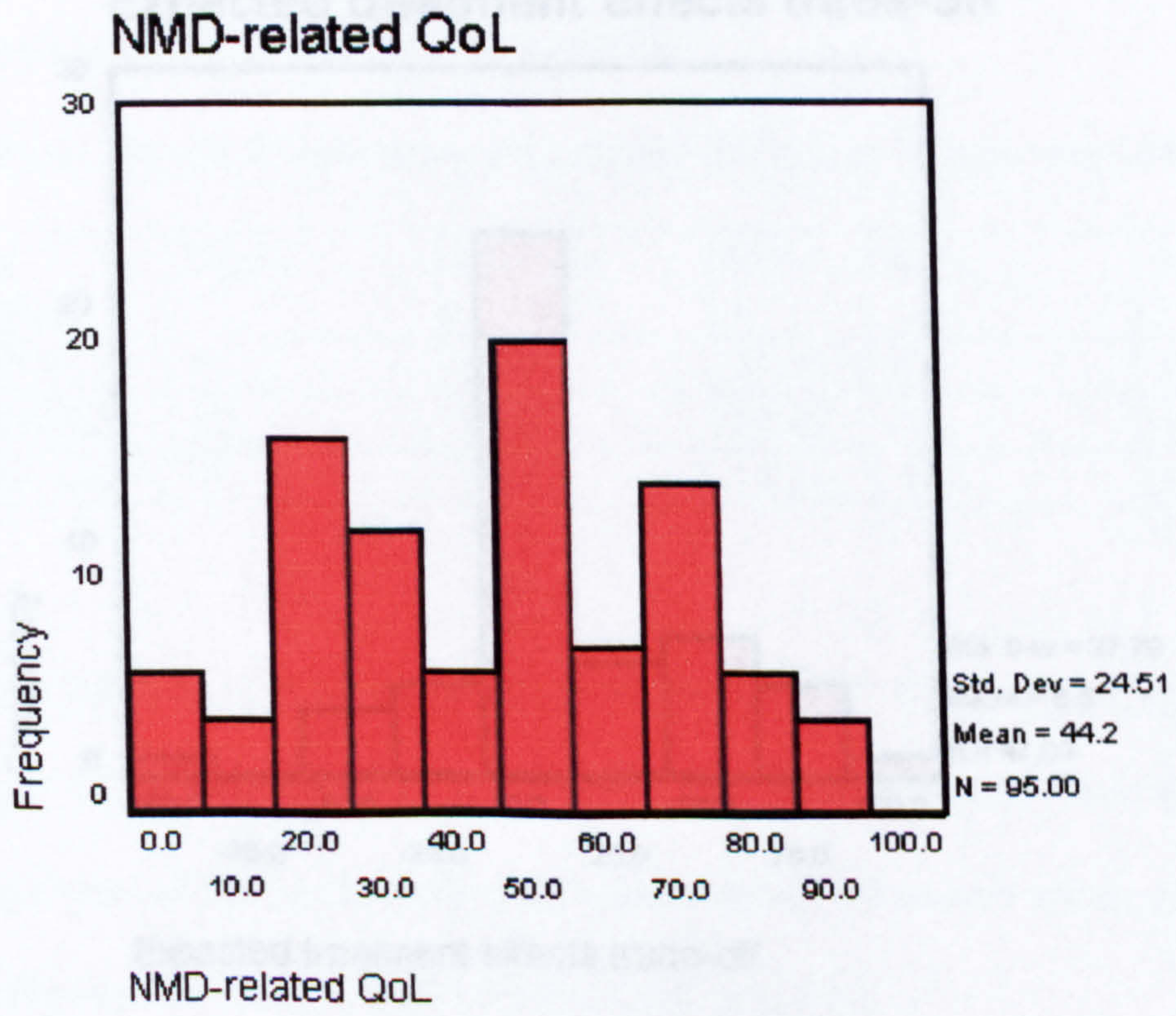


Figure 11.11: Distribution of treatment effects trade-off scores

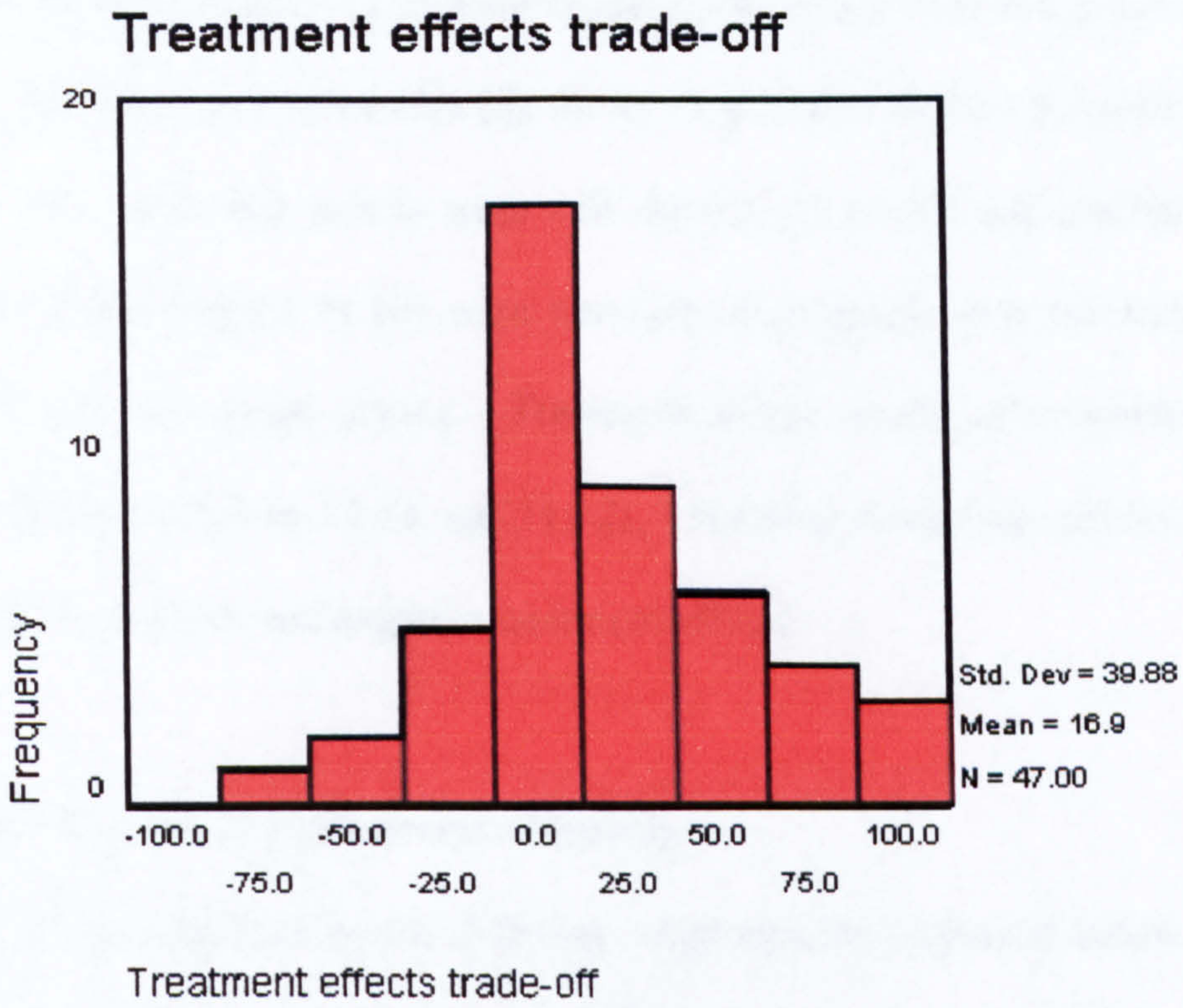
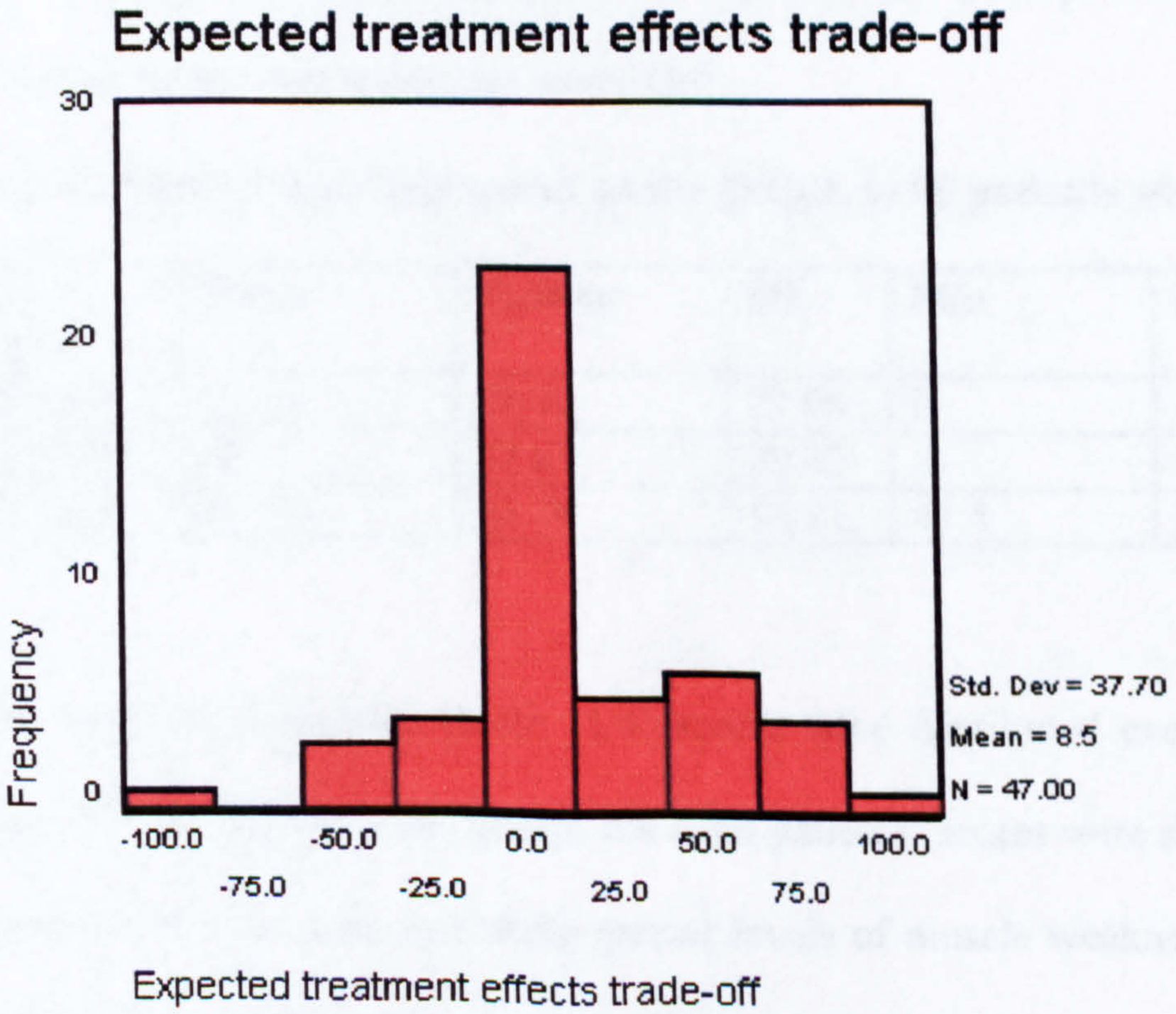


Figure 11.12: Distribution of expected treatment effects trade-off scores



Scores on the Activities (figure 11.5), Emotions (figure 11.8) and Body Image (figure 11.9) dimensions were evenly distributed across the range, but scores on Independence (figure 11.6) were slightly skewed towards the lower end of the scale. The Relationships dimension (figure 11.7) was also skewed towards the lower end of the scale, although scores were still distributed across the spectrum. NMD-related QoL scores (figure 11.10) were normally distributed, with the majority of patients obtaining mid-range scores. Treatment effect scores demonstrated a bell-shaped distribution (figures 11.11 and 11.12), indicating most respondents to report similar levels of positive and negative treatment effects.

11.1.2.2 Score profiles across subgroup

The scores obtained by the different subgroups, as displayed below in tables 11.4 to 11.15, demonstrate the INQoL's ability to reflect differing profiles of disease impact. Patients in the ASP group demonstrated higher mean scores on many of the dimensions, demonstrating a greater negative impact of their NMD upon these areas of life. This suggests that these patients have poorer QoL than patients in the other subgroups as higher scores indicate worse QoL.

Table 11.4: Muscle Weakness scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	57.71	68.42	27.06	0	100
ARR (N=50)	50	52.63	29.49	0	100
ASP (N=10)	67.37	65.79	14.21	47.4	94.7

For the weakness dimension (table 11.4) scores were distributed evenly across the spectrum for the CSP and ARR groups but ARR patients' scores were skewed towards the upper end of the scale, indicating greater levels of muscle weakness. The lowest score obtained by patients with an ASP NMD was 47.4.

Table 11.5: Muscle 'locking' scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	28.29	21.06	29.23	0	68.42
ARR (N=50)	9.92	0	21.69	0	94.74
ASP (N=10)	0	0	0	0	0

A large proportion of respondents did not report experiencing muscle 'locking' (table 11.5). None of the patients in the ASP group and only a very small number of the ARR group reported this symptom, which unsurprising as this symptom is not a recognised feature of these conditions.

Table 11.6: Pain scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	19.08	0	28.26	0	73.68
ARR (N=50)	31.84	21.05	31.42	0	100
ASP (N=10)	25	21.05	25.55	0	57.89

The mean and median scores for pain (table 11.6) indicate that patients tend to experience this symptom less intensely than they experience muscle weakness or fatigue.

Table 11.7: Fatigue scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	44.73	50	40.66	0	89.47
ARR (N=50)	50	52.63	29.49	0	100
ASP (N=10)	51.32	47.37	21.65	31.58	78.95

The fatigue scores (table 11.7) demonstrate more of a spread over the range of possible scores, although ARR patients did not score at either the extreme low or extreme high ends of the scale.

Table 11.8: Activities scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	46.64	33.33	28.47	21.30	97.22
ARR (N=50)	46.03	47.22	30.35	0	97.22
ASP (N=10)	67.13	69.45	25.00	39.81	89.81

Patients demonstrated activities scores ranging across the scale from low to high. However patients in the CSP and ASP groups did not score at lowest end of the scale indicating that all the patients with these muscle conditions were influenced in their activities in some way. The ASP group in particular had high scores for negative impact upon their activities.

Table 11.9: Independence scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	35.42	30.56	36.45	0	86.11
ARR (N=50)	34.34	30.56	28.85	0	86.11
ASP (N=10)	59.03	59.72	6.94	50	66.66

The range of Independence scores in the CSP and the ARR subgroups of patients (table 11.9) suggest that the INQoL captures a wide range of independence levels. ASP patients demonstrated scores within a more limited range.

Table 11.10: Social Relationships scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	25.81	28.71	15.51	0	47.22
ARR (N=50)	22.50	23.15	17.74	0	60.19
ASP (N=10)	35.42	30.56	24.46	12.96	67.59

The Social Relationship subscale was more skewed towards the lower end of the scale, with patients perceiving there to be less of an impact upon their Social Relationships than upon other life domains incorporated in the scale.

Table 11.11: Emotions scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	48.26	43.06	34.02	8.33	91.67
ARR(N=50)	34.39	33.33	22.27	0	92.59
ASP (N=10)	47.92	40.28	22.27	30.56	80.56

The Emotions subscale demonstrates a wide variation in scores, although the ASP group scores were not as widely distributed.

Table 11.12: Body Image scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	43.06	40.28	35.07	0	97.22
ARR (N=50)	43.76	41.67	30.50	0	97.22
ASP (N=10)	55.56	58.34	23.35	25.00	80.56

The Body image subscale again demonstrated a wide distribution of scores (table 11.12). Average scores fell around the mid-point of the scores possible.

Table 11.13: NMD-related quality of life scores on the INQoL in 95 patients with NMD

Patient subgroup	Mean	Median	SD	Min	Max
CSP (N=35)	41.60	33.61	27.23	11.67	87.22
ARR(N=50)	42.82	48.33	25.38	0	83.33
ASP (N=10)	60.97	53.61	17.73	49.44	87.22

Scores for NMD-related quality of life (table 11.13) were spread across the range of scores possible and average scores were close to 50 (or the mid-point on the scale).

11.1.3 Construct validity

Table 11.2 shows the characteristics of the patients taking part in testing the validity of the INQoL.

Hypothesis 1: Functional disability

Patients with more functional disability will report NMD to have a greater impact upon their activities and their independence and will give higher ratings of symptom impact.

Of the patients attending for an appointment, 32 completed the 'timed walk' and 29 the 'timed stands' task. As shown in table 11.14 there was a strong correlation between both the Activities and the Independence scores and scores on the functional tests.

Table 11.14: Relationships between the dimensions of Activities and Independence and the timed functional tests

Test	Activities		Independence	
	Spearman's rho	p value	Spearman's rho	p value
Timed 10m walk	0.45	0.006	0.67	0.000
Timed stands	0.76	0.000	0.82	0.000

Tables 11.15 to 11.17 show the correlation coefficients found between individual symptom scores and functional tests. The muscle weakness scores demonstrated a strong correlation with the functional tests. There were no significant relationships between any of the other symptom scores and times on the functional tests.

Table 11.15: Relationship between the dimensions of Muscle Weakness and Fatigue and the timed functional tests

Test	Muscle Weakness		Fatigue	
	Spearman's rho	p value	Spearman's rho	p value
Timed 10m walk	0.59	0.000	0.29	0.07
Timed stands	0.77	0.000	0.20	0.14

Table 11.16: Relationship between the dimensions of Muscle 'Locking' and pain and performance on the timed functional tests

Test	Muscle 'Locking'		Pain	
	Spearman's rho	p value	Spearman's rho	P value
Timed 10m walk	0.11	0.395	0.32	0.038
Timed stands	-0.05	0.282	0.26	0.088

As only 17 of the 32 patients reported muscle 'locking' and just 20 reported pain, a subgroup analysis was performed for these two symptoms. The subgroup analysis yielded higher correlation coefficients between these symptoms and the functional tasks, but only the pain score and timed stands correlation reached significance (tables 11.17 and 11.18).

Table 11.17: Relationship between muscle 'locking' scores and timed functional tests in patients reporting muscle 'locking'

		Muscle 'Locking'	
	N	Spearman's rho	p value
Timed 10m walk	17	0.31	0.11
Timed stands	15	0.19	0.252

Table 11.18: Relationship between pain scores and timed functional tests in patients reporting pain

		Pain	
	N	Spearman's rho	p value
Timed 10m walk	20	0.18	0.218
Timed stands	17	0.45	0.04

Hypothesis 2: Symptoms

Patients who rate their NMD symptoms as more severe will report these symptoms to have a greater impact upon their lives

Table 11.19 shows the Spearman's rank correlation coefficients between the symptom subscales of the questionnaire and the validated reference measures. The hypothesis was supported with concordance between the VASs for pain, weakness and muscle 'locking' and the corresponding subscales of the INQoL.

The relationship between INQoL Fatigue scores and Chalder fatigue scores was weaker than the relationships demonstrated between the other symptoms and their reference measures.

Table 11.19: Relationships between symptom subscores of the INQoL and validated measures of these symptoms

New questionnaire dimension and validation scale	Spearman's rho	p value
Muscle weakness score and weakness VAS	0.76	0.000
Muscle 'locking' score & Locking VAS	0.69	0.000
Pain score & Pain VAS	0.75	0.000
Pain score & SF36 Pain	-0.78	0.000
Fatigue score & Chalder Fatigue score	0.49	0.000
Fatigue score & Chalder physical fatigue score	0.51	0.000

Hypothesis 3: Activities

Patients reporting difficulties with mobility and self-care tasks will report more difficulty in carrying out physical activities and greater dissatisfaction with their ability to carry out their activities.

The hypothesis was fulfilled with a reasonably strong relationship (Spearman's Rho= -0.59; p= 0.000) between the Activities score of the INQoL and the Physical Functioning scale of the SF-36.

Hypothesis 4: Independence

Patients reporting difficulties with mobility and self-care tasks will report lower levels of independence and greater dissatisfaction with their degree of independence.

This hypothesis was fulfilled by a strong relationship (r= 0.67; p= 0.000) between Independence scores and scores on the Barthel index.

Hypothesis 5: Social Relationships

Patients who have less social support will:

Ia. experience greater difficulties in social relationships.

Ib. report a greater impact of NMD upon their quality of life.

Patients reporting difficulties in social relationships will also:

IIa. report more of an influence of NMD upon their relationships and greater dissatisfaction with these relationships.

As displayed in table 11.20, the SSQ6 demonstrated a modest but significant correlation with the Social Relationships dimension of the INQoL. The social impact domains of the SF-36, FLP and the NHP demonstrated much stronger relationships with the social dimension of the INQoL. Scores on the SSQ6 also demonstrated a modest relationship with NMD-related QoL scores on the INQoL.

Table 11.20: Relationships between INQoL Social Relationships score and validated measures of social support and social functioning

New questionnaire dimension and validation scale	Spearman's rho	p value
Relationships score & SSQ Number score	-0.29	0.009
Relationships score & SSQ Satisfaction score	-0.34	0.003
Relationships score & SF36 Social functioning	-0.61	0.000
Relationships score & FLP Social Interaction	0.66	0.000
Relationships score & NHP Social Isolation	0.54	0.000
QoL score & SSQ Number score	-0.37	0.002
QoL score & SSQ Satisfaction score	-0.31	0.009

Hypothesis 6: Emotions

Patients exhibiting high levels of depression and anxiety will report a greater impact of NMD upon their emotions and will be more dissatisfied with their emotional well-being.

The Emotions subscale score of the INQoL demonstrated a strong relationship with the Anxiety and Depression dimensions of the HAD scale and with the corresponding scales of the SF-36, the FLP and the NHP (Table 11.21).

Table 11.21: Relationships between Emotions score on the INQoL and validated measures of emotion

New questionnaire dimension and validation scale	Spearman's rho	p value
Emotions score & HADS Anxiety	0.63	0.000
Emotions score & HADS Depression	0.66	0.000
Emotions score & SF36 Mental health	-0.55	0.000
Emotions score & FLP Emotion	0.71	0.000
Emotions score & NHP Emotional Reactions	0.66	0.000

Hypothesis 7: Body Image

Patients with a more negative body image will report more of an impact of NMD upon how they feel about their physical appearance.

This was fulfilled by a strong relationship between the Body Image dimension scores with both dimensions of the ABESr (table 11.22).

Table 11.22: Relationships between the Body Image dimension of the INQoL and validated measures of Body Image

New questionnaire dimension and validation scale	Spearman's rho	p value
Body image score & ABESr (Body Totality)	-0.68	0.000
Body image score & ABESr (Body Self-consciousness)	-0.57	0.000

Hypothesis 8: Quality of Life

Patients with low levels of QoL as measured by generic QoL scales will have more negative QoL as measured by the INQoL.

As predicted, the QoL scores derived from the FLP and from the PGI demonstrated strong concordance with the NMD-related QoL score on the new questionnaire (Table 11.23).

Table 11.23: Relationship between QoL score on the INQoL and validated measures of QoL

New questionnaire dimension and validation scale	Spearman's rho	p value
NMD-Related QoL & FLP Total score	0.76	0.000
NMD-Related QoL & PGI	-0.57	0.000

There was also a considerable overlap between the domains generated by patients for the PGI and those domains incorporated in the INQoL (table 11.24). Patients also generated other more specific life areas not specifically mentioned in the new questionnaire (e.g. holidays, safety, and confidence).

Table 11.24: Domains listed by the patients in the PGI that correspond to the domains in the new questionnaire

Domain listed	N listing life area	INQoL dimensions incorporating domain
Work	20	Activities
Social life/socialising	18	Activities
Social/leisure activities	10	Activities
Sport (including: football, squash, outdoor sports)	17	Activities*
Walking	13	Activities*
Holidays/Travel	11	Activities*
Mobility	6	Activities*
Specific leisure activities (e.g. dancing, walks)	5	Activities*
Stairs	5	Activities*
Physical activities	5	Activities*
Independence	5	Independence
Family	5	Relationships
Cooking/housework	5	Activities
Falls	5	N/A
Relationships	5	Relationships
Home	4	N/A
Emotions (including depression & worry)	4	Emotions
Shopping	4	Activities*
Bathing	4	Activities*
Tiredness/Energy	3	Symptoms
Strength	3	Symptoms
Physical changes/ Appearance	3	Body Image
Fitness	3	N/A
Grandchildren	3	Relationships*
Relationships with spouse/partner	3	Relationships
Lifting	3	Activities*
Standing, getting up from chair	3	Activities*
Eating food/swallowing	2	Activities*
Rash	2	Body image*
Other people's social behaviour / General public	2	Relationships*
Confidence	2	Emotions*
Balance	2	N/A
Other 'life areas' listed *Sleep, Looking after children, Safety, Grip, Choking, Driving, Rolling own cigarettes, Close friendships, Diet, Future, Public transport, Daily living, Sleeping, Treatment, Physical well-being, ability to use hands, religion, academic, building, safety, freedom, eating, feeling cold, clothing, trying to stay motivated, Personal qualities, Use of brain, Acceptance of problem*	1	Various domains

* Indicates 'domain' covered but not specifically mentioned in the INQoL

N/A Signifies domain not included in the INQoL

11.1.4 Reliability

Of the 46 people asked to participate in the test-retest reliability study, 40 patients took part, completing the form on a second occasion, one week after the initial administration (see table 11.2 for a description of patient characteristics).

The scatterplots depicted in figures 11.13 to 11.22 show the agreement between scores on the individual scales at initial completion of the scale and at retest. 95% confidence intervals were calculated for all the dimensions and these upper and lower limits of agreement are marked on the graph.

Table 11.25: Agreement between the two administrations of the questionnaire in 40 NMD patients

INQoL Domain	Mean change in INQoL score	SD of the mean change in INQoL subscale score	Upper limit of agreement	Lower limit of agreement
Muscle weakness	5.62	14.15	33.35	-22.68
Muscle 'locking'	-0.0003	14.55	28.52	-28.52
Pain	1.6	19.52	39.86	-36.66
Fatigue	-1.05	19.52	37.21	-39.31
Activities	1.82	14.15	29.55	-25.91
Independence	0.19	15.24	30.06	-29.68
Relationships	-1.68	13.63	25.03	-28.39
Emotions	0.25	12.93	25.59	-25.09
Body Image	4.12	18.80	40.97	-32.73
NMD related QoL	-0.34	12.87	24.89	-25.57

The test-retest reliability of the scale is demonstrated by the mean difference between the INQoL domain scores at initial test and retest, the standard deviation (SD) of the mean change in INQoL scores and upper and lower levels of agreement (table 11.25).

All but two of the ten subscales demonstrated a mean change of less than 2 points for their mean score, demonstrating a good level of stability. Out of the ten subscales, seven demonstrated a mean score that was slightly higher on retest, indicating a slight worsening of scores from initial test to retest. Nonetheless, seven of the ten subscales demonstrated limits of agreement that were less than 35 points from zero (point of no change).

Figures 11.13 and 11.14 demonstrate that the scores obtained for Weakness and Muscle 'Locking' on the first and second administration of the scale demonstrated acceptable levels of agreement. For Pain and Fatigue the distribution of the difference scores was wider with a SD of 19.52 on both scales (figures 11.15 & 11.16).

Scores on the Activities (figure 11.17), Independence (figure 11.18), Social Relationships (figure 11.19) and Emotions (figure 11.20) subscales at initial test and retest also demonstrated good levels of agreement with upper and lower limits of agreement less than 30 points away from 0 (0= perfect agreement). Scores on the Body Image subscale (figure 11.21) demonstrated slightly lower levels of agreement with differences demonstrating a SD of 18.8.

Figure 11.13: Distribution of differences between 1st and 2nd Weakness scores

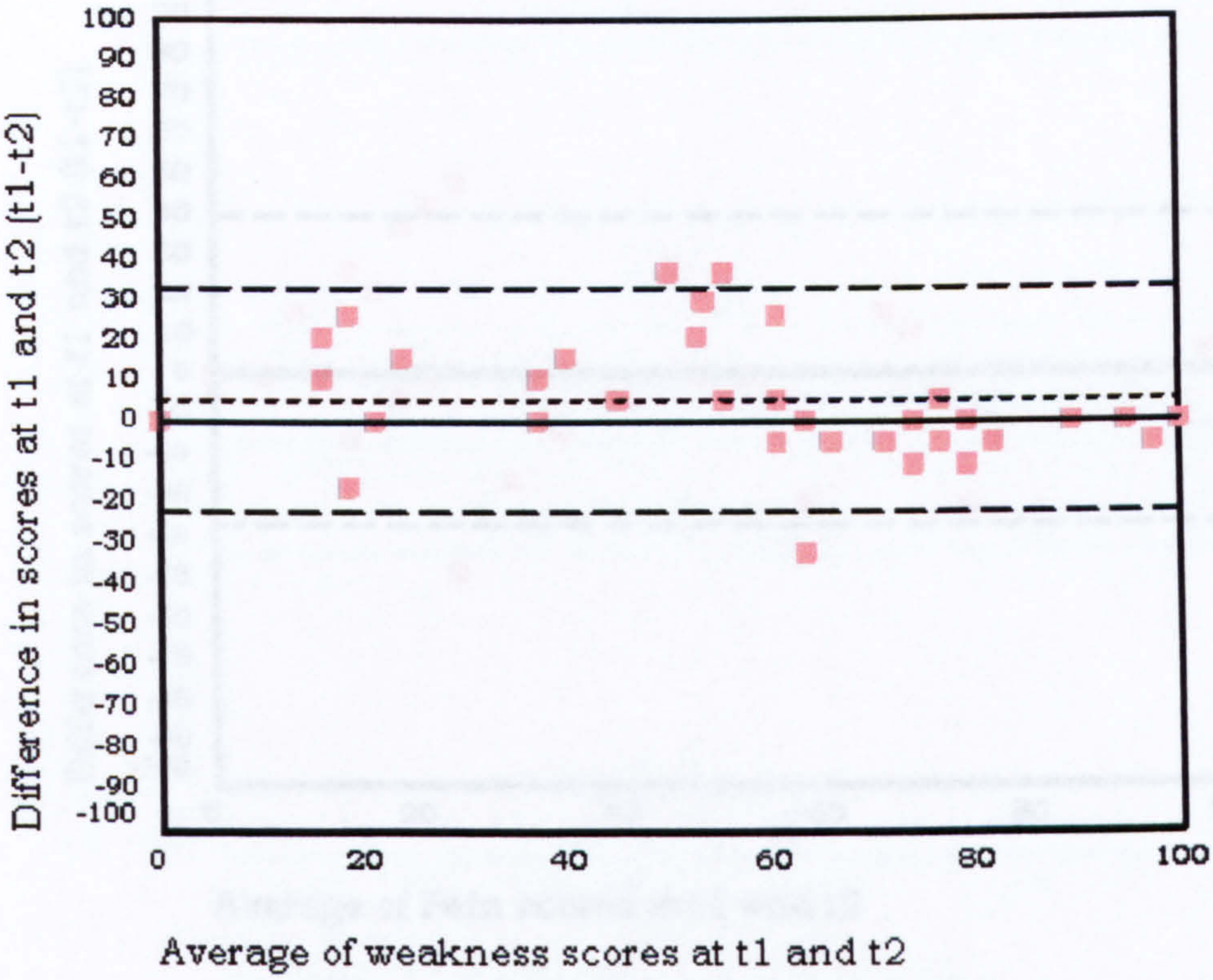


Figure 11.14: Distribution of differences between 1st and 2nd 'Locking' scores

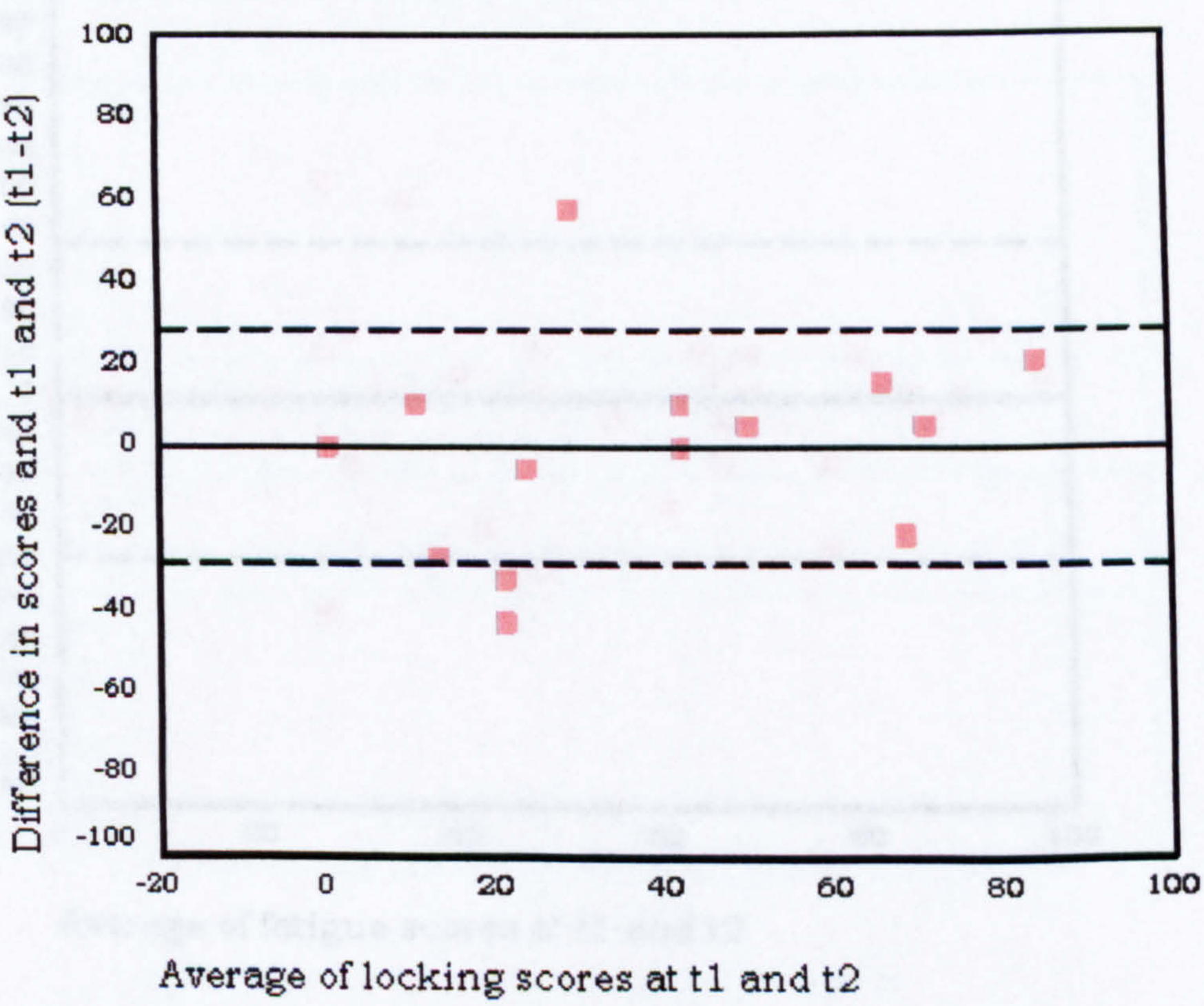


Figure 11.15: Distribution of differences between 1st and 2nd Pain scores

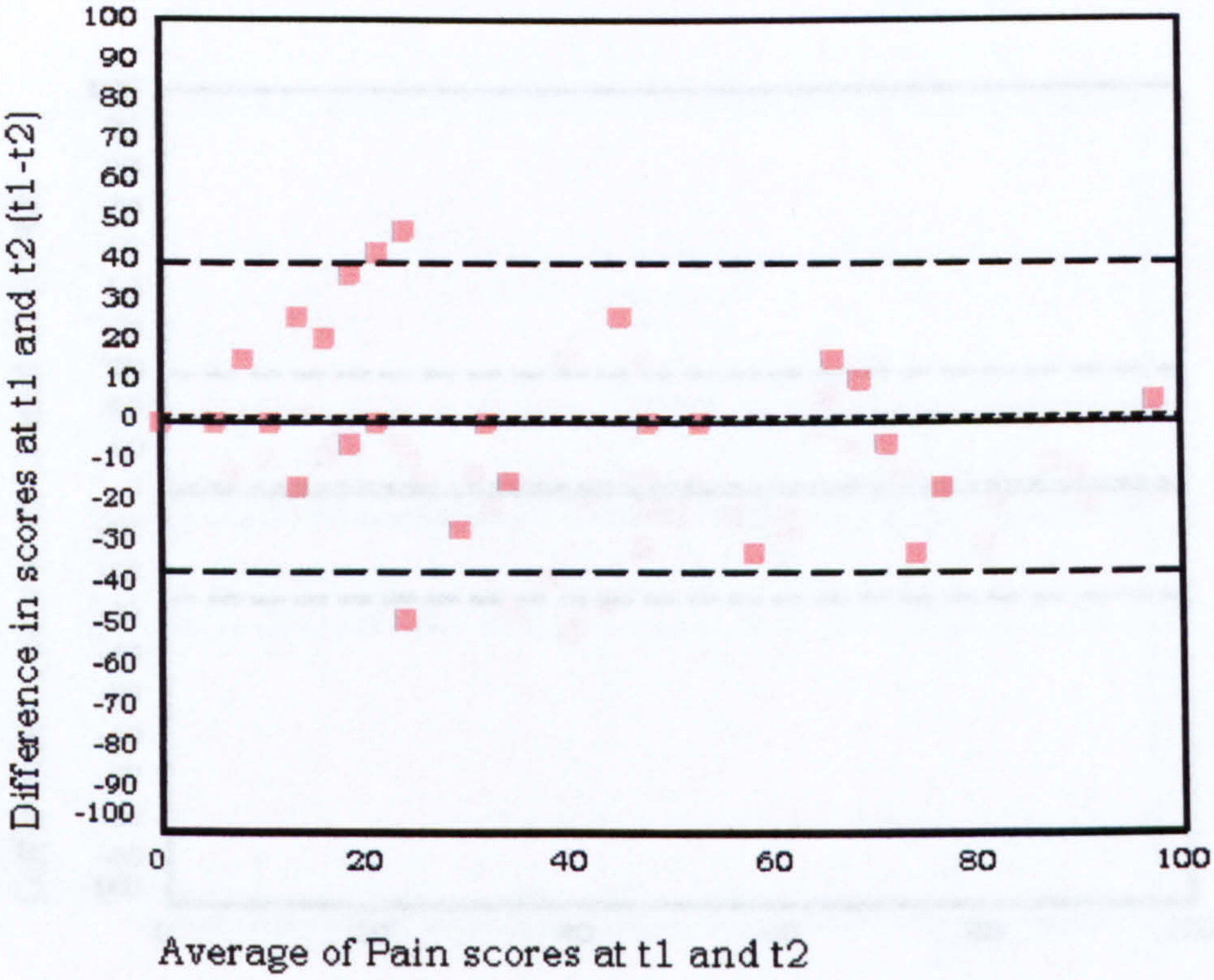


Figure 11.16: Distribution of differences between 1st and 2nd Fatigue scores

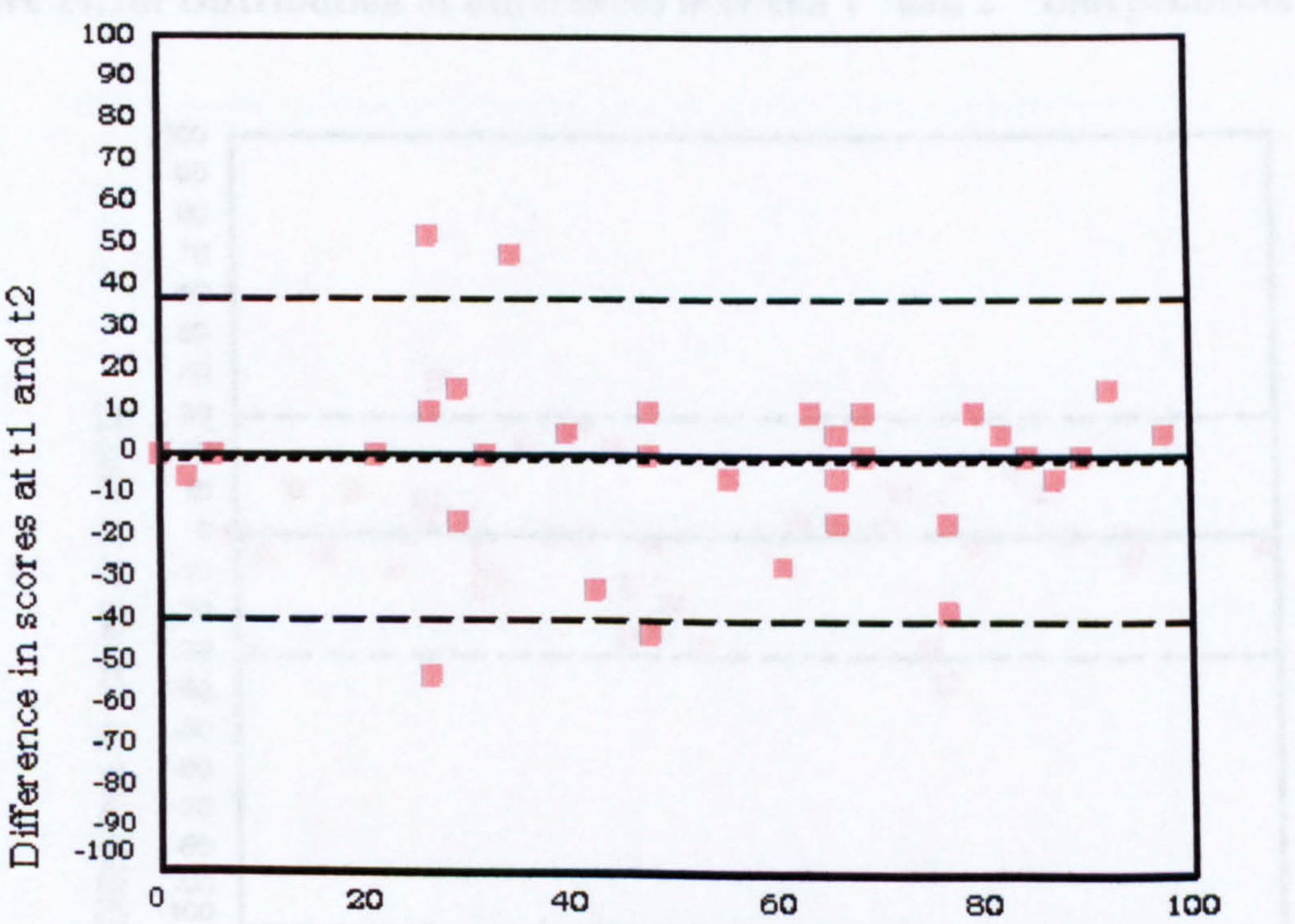


Figure 11.17: Distribution of differences between 1st and 2nd Activities scores

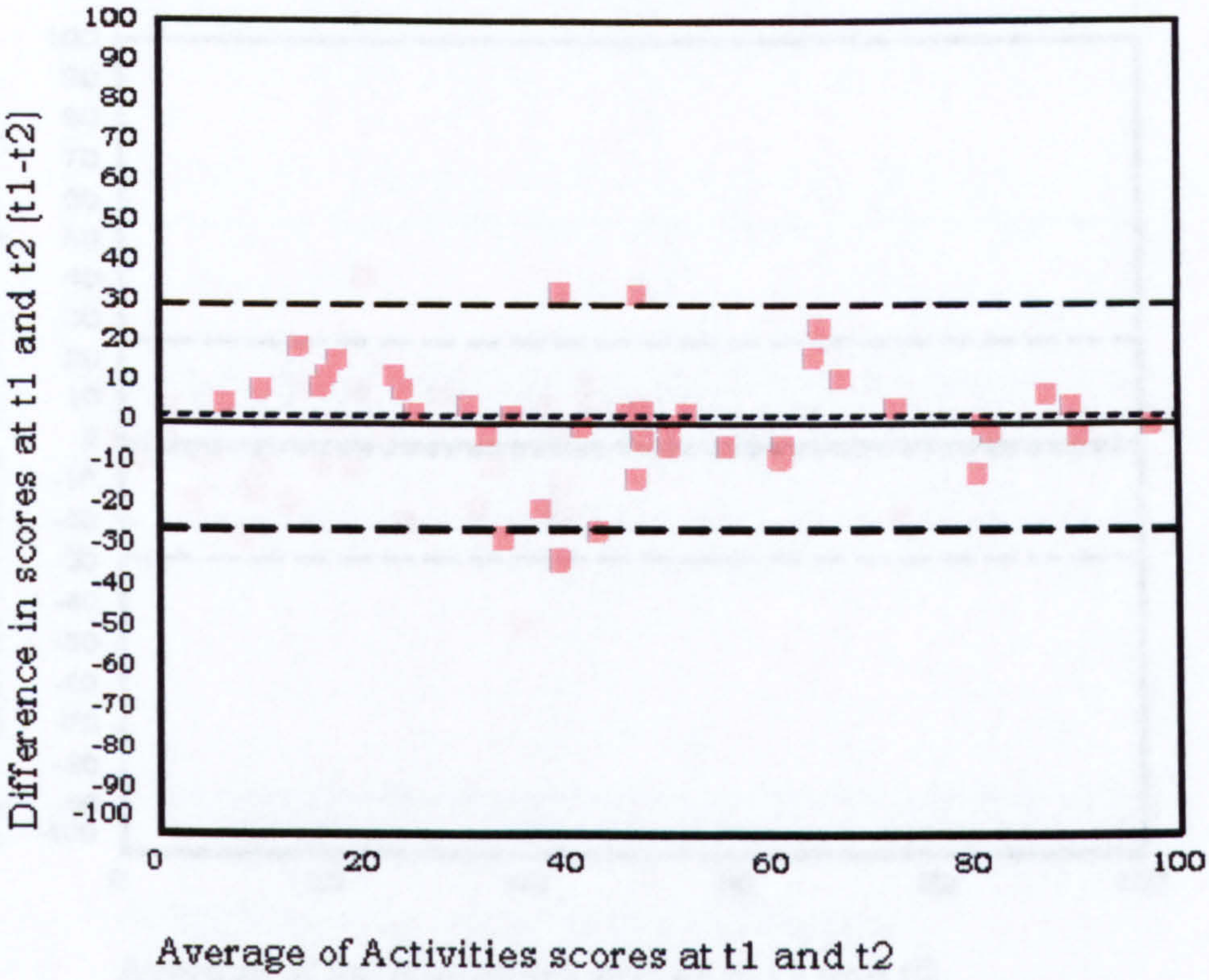


Figure 11.18: Distribution of differences between 1st and 2nd Independence scores

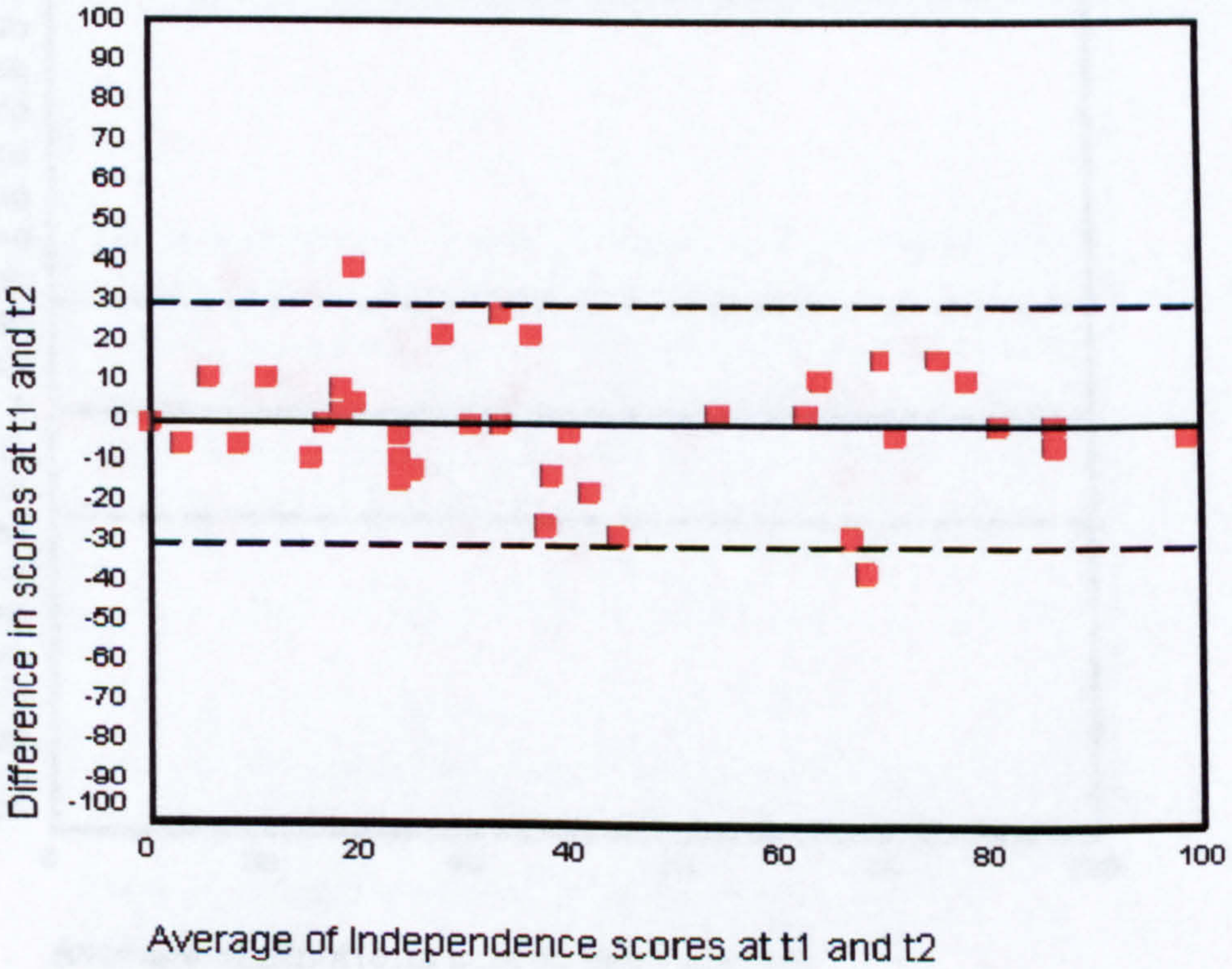


Figure 11.19: Distribution of differences between 1st and 2nd Relationships scores

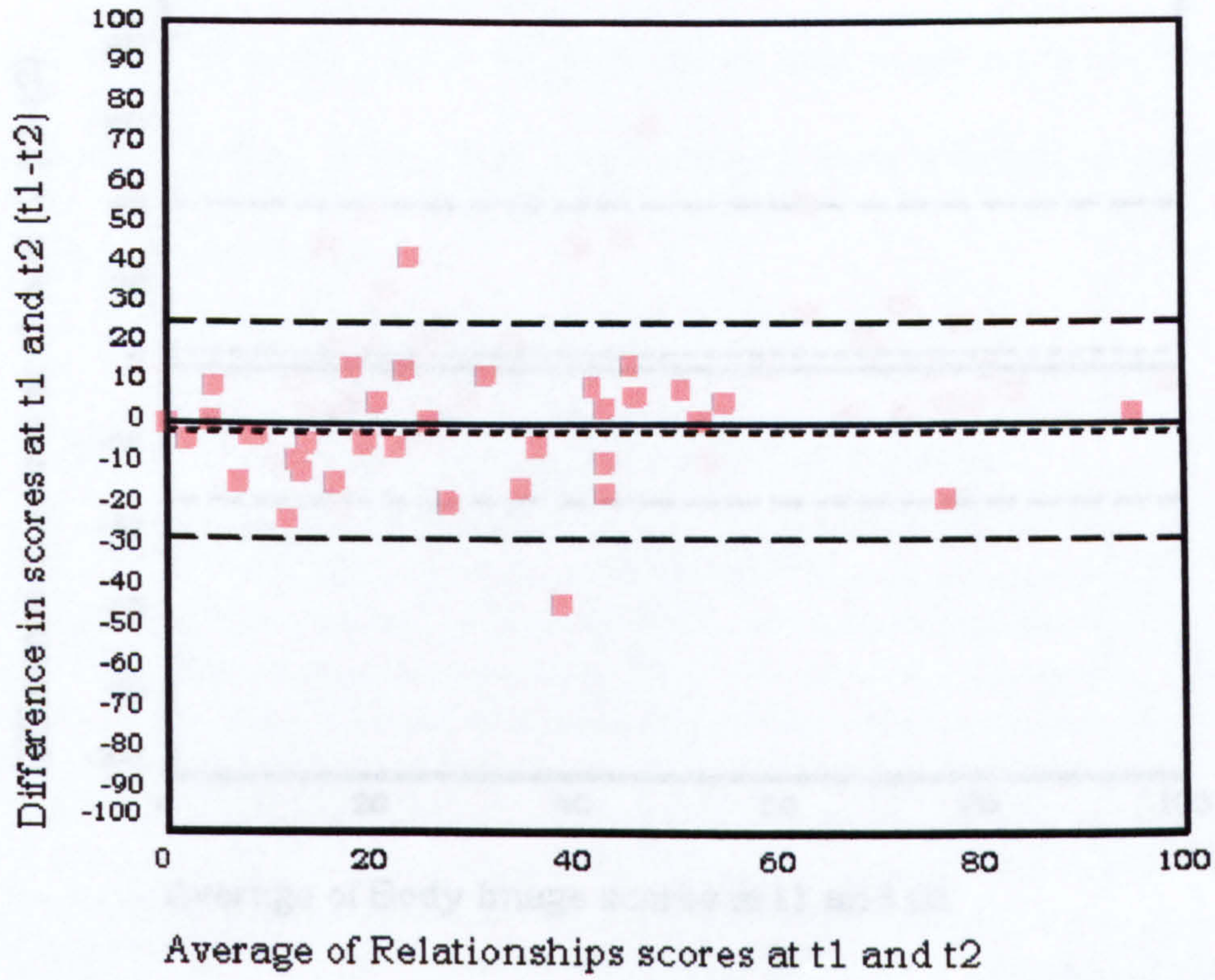


Figure 11.20: Distribution of differences between 1st and 2nd Emotions scores

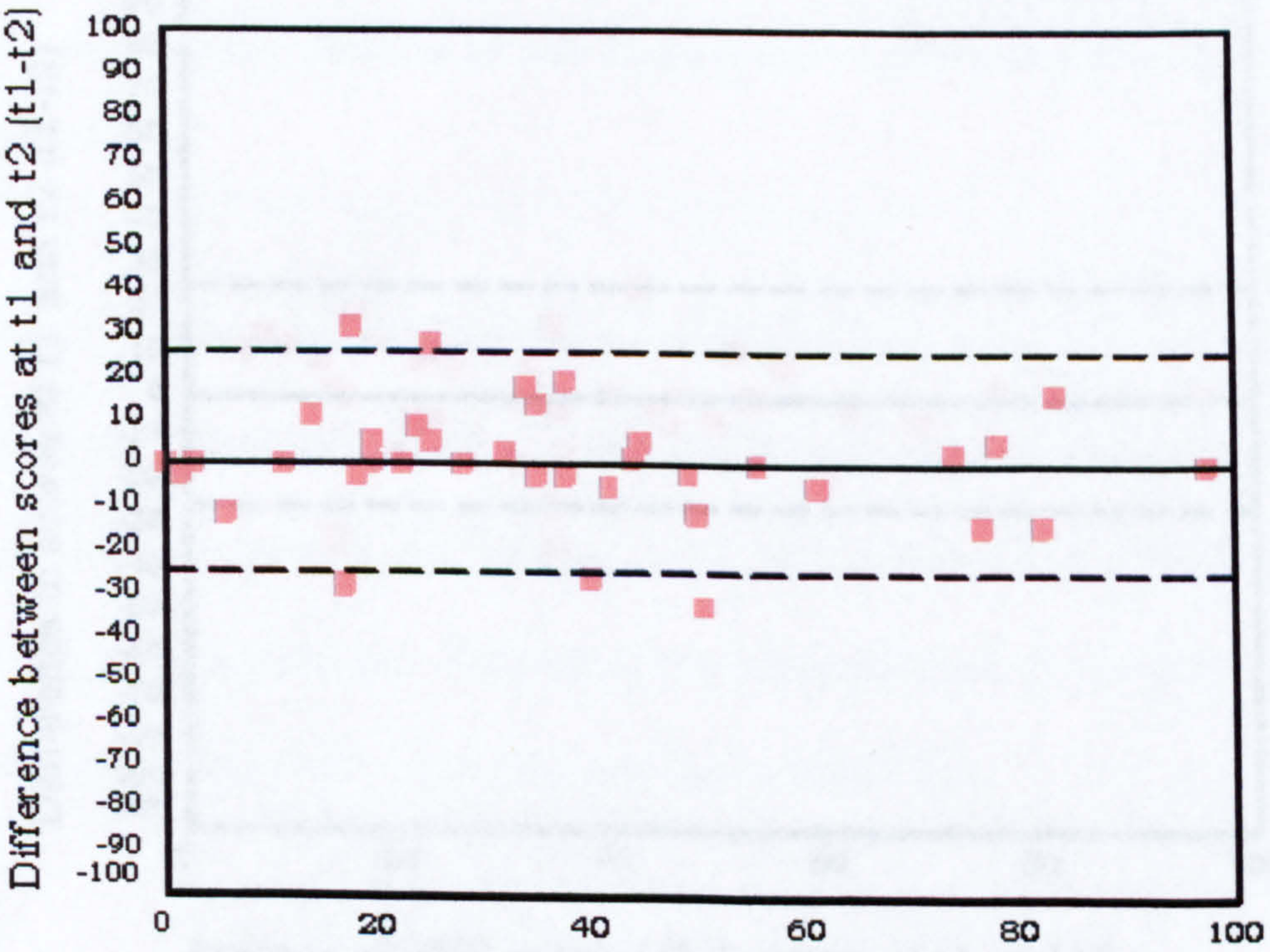


Figure 11.21: Distribution of differences between 1st and 2nd Body image scores

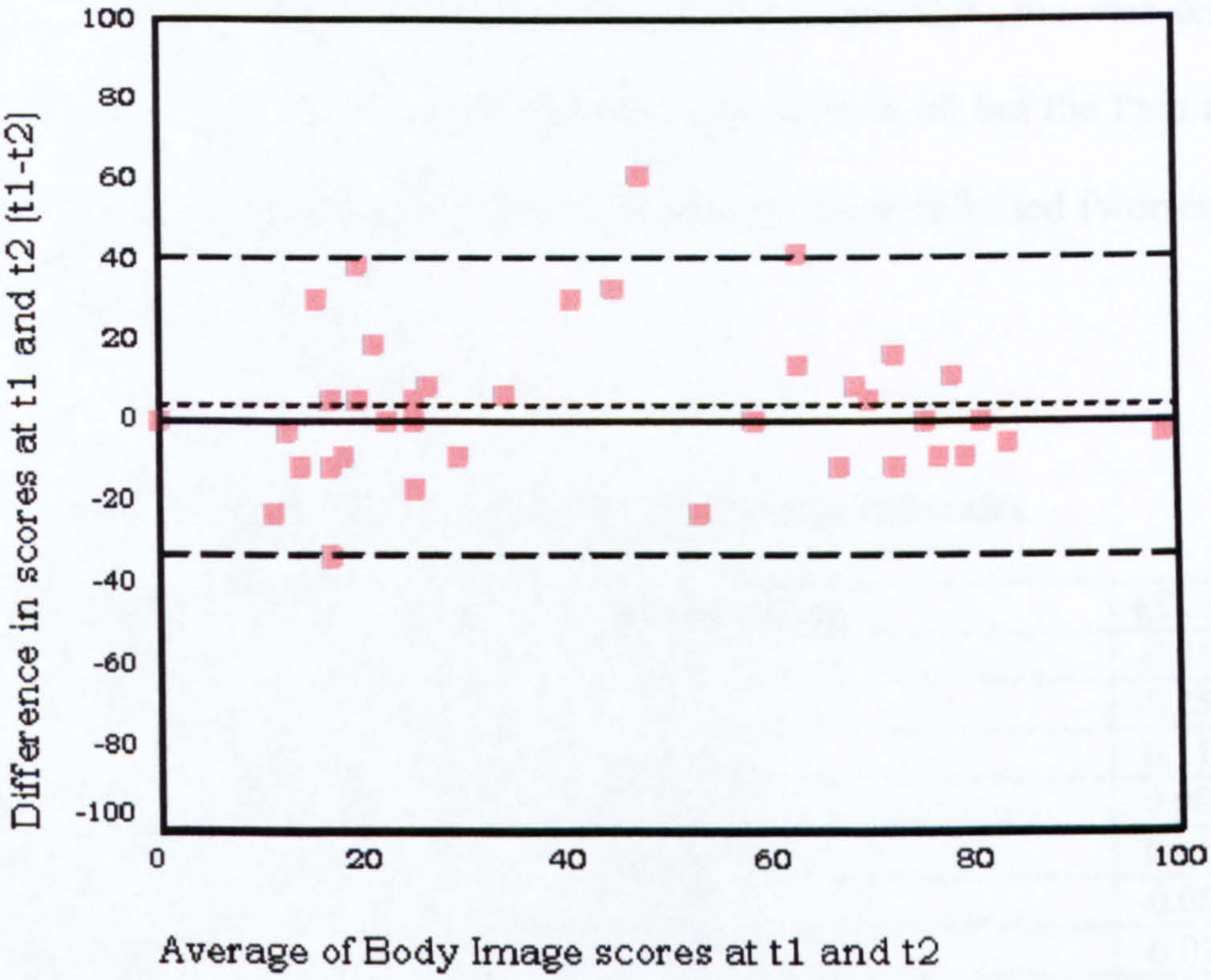
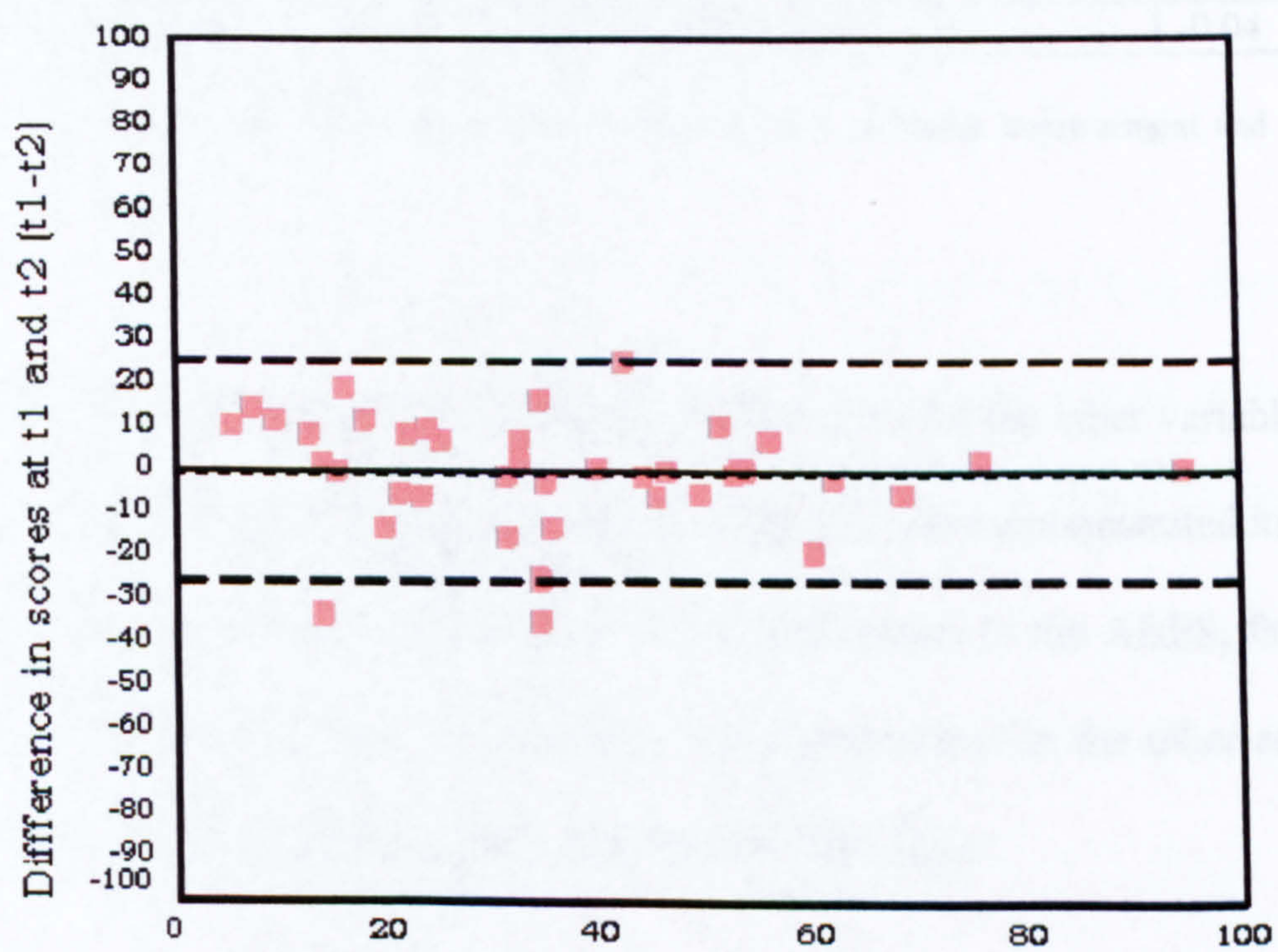


Figure 11.22: Distribution of differences between 1st and 2nd NMD-related QoL scores



11.1.5 Responsiveness

Table 11.26 shows the mean changes in each of the scale dimensions over the 3-6 month period in which the responsiveness of the questionnaire was tested. These changes were very small with negligible effect sizes in all but the Pain and Emotion dimensions. In these dimensions, small changes were reflected (worsening of Pain, improvement in Emotions).

Table 11.26: Effect sizes demonstrated by INQoL subscales

Questionnaire Dimension	Mean change	Effect size
Muscle weakness	-0.16	-0.01
Muscle 'locking'	-4	-0.15
Pain	**6.32**	**0.21**
Fatigue	2.96	0.08
Symptoms	0.22	0.01
Activities	0.09	-0.00
Independence	-0.44	-0.01
Relationships	-0.43	-0.02
Emotions	**-6.89**	**-0.24**
Body Image	-0.43	-0.02
Negative impact of NMD	-1.49	-0.06
NMD related QoL	-1.09	-0.04

† Bold print indicates effect size of note. Negative score indicates improvement and positive score indicates decline.

Table 11.27 shows the mean changes and effect sizes for the other variables measured at both time points. Effect sizes of small magnitude were demonstrated in some of the other measures, namely the 'locking' VAS, both scales of the ABES, fatigue scores, and the timed stands test. The effect sizes demonstrated in the other scales did not correspond to the effect sizes demonstrated by the INQoL.

Table 11.27: Effect sizes of the various reference measures

Variable	Mean change	Effect size
Barthel	0.2 *(I)*	0.07
Weakness VAS	1.08 *(D)*	0.04
Pain VAS	2.56 *(D)*	0.10
'Locking' VAS	**7.12 *(D)***	**-0.28**
Patient Generated Index	3.18 *(D)*	0.11
ABES (Body totality)	**2.64 *(I)***	**0.312**
ABES (Body self-consciousness)	**-2.12 *(D)***	**-0.216**
Fatigue score	**1.84 *(D)***	**-0.34**
Physical fatigue	**1.4 *(D)***	**-0.32**
Mental fatigue	**1.08 *(D)***	**-0.44**
Timed walk	1.16 *(D)*	-0.12
Timed stands	**4.87 *(D)***	**-0.18**
SF36 Physical functioning	0.60 *(I)*	0.02
SF36 Role Physical	-4 *(D)*	-0.09
SF36 Role Emotional	5.35 *(I)*	0.13
SF36 Social Functioning	4.93 *(I)*	0.17
SF36 Mental Health	-0.92 *(D)*	-0.06
SF36 Energy/Vitality	-3.4 *(D)*	-0.15
SF36 Pain	0.88 *(I)*	0.03
SF36 General Health Perceptions	-2.64 *(D)*	-0.13

† Bold print indicates effect size of note, *I*= Improvement, *D*= Decline

Patients who completed the questionnaire were divided into three groups based on whether they reported an improvement, deterioration or no change in their condition since their first appointment. The characteristics of the patients within these three subgroups are reported in table 11.28.

Table 11.28: Characteristics of the sub-samples taking part in the responsiveness study

Subsamples	Male female ratio	Mean age	Patient subgroup
Worse (N=7)	1.33 : 1	56 (SD 10.95 min-max 32-63)	6 CSP 1 ASP
Better (N=4)	1 : 3	49 (SD 0.5 min-max 38-64)	1 CSP 3 ARR
Same (N=14)	1.33 : 1	49 (SD 14.7 min-max 28-77)	12 CSP 1 ARR 1 ASP

This enabled an analysis of effect sizes in patients who would be expected to demonstrate a change in score corresponding to a reported change in their condition compared to patients who reported no change (table 11.29).

Effect sizes on a number of the subscales demonstrated changes in the expected direction. In the four patients who reported improvement there were small effect sizes indicating improvements in fatigue, activities, independence and NMD-related quality of life and a moderate effect size reflecting improvement in emotions.

In the patients who stayed the same there were small effect sizes in three of the subscales. These indicated a small improvement in emotions but deterioration in pain and fatigue scores.

For the seven patients reporting deterioration in their condition, the Pain scale demonstrated a moderate effect size in the expected direction (decline = increased pain).

Table 11.29: Effect sizes in dimensions of the INQoL by subgroups representing patients who reported either an improvement, a deterioration or no change in their condition

Questionnaire Dimension	Effect size in patients reporting an improvement (4 patients)		Effect size in patients reporting staying the same (14 patients)		Effect size in patients reporting an deterioration (7 patients)	
	Mean change	Effect size	Mean change	Effect size	Mean change	Effect size
Muscle weakness	-2.63 *(I)*	-0.09	2.35 *(D)*	0.08	-3.76 *(I)*	-0.21
Muscle 'locking'	-5.26 *(I)*	-0.5	-4.13 *(I)*	-0.15	-3.01 *(I)*	-0.10
Pain	-2.63 *(I)*	-0.09	**6.77** *(D)*	**0.21**	**10.53***(D)*	**0.40**
Fatigue	**-7.85** *(I)*	**-0.20**	**6.28** *(D)*	**0.19**	1.51 *(D)*	0.05
Symptoms	-4.61 *(I)*	-0.17	2.44 *(D)*	0.10	-1.46 *(I)*	-0.09
Activities	**-6.71** *(I)*	**-0.24**	0.69 *(D)*	0.02	2.78 *(D)*	0.12
Independence	**-3.48** *(I)*	**-0.21**	-1.79 *(I)*	-0.05	3.97 *(D)*	0.13
Relationships	-2.32 *(I)*	-0.15	0.55 *(D)*	0.02	-1.32 *(I)*	-0.08
Emotions	**-16.99** *(I)*	**-0.56**	**-6.81** *(I)*	**-0.26**	-1.19 *(I)*	-0.04
Body Image	0.69 *(D)*	0.05	-3.17 *(I)*	-0.11	4.42 *(D)*	0.13
NMD-related QoL	**-5.57**	**-0.22**	-0.40 *(I)*	-0.02	0.09 *(D)*	0.005

† Bold print indicates effect size of note. Negative score indicates improvement and positive score indicates decline.

Table 11.30 lists the effect sizes demonstrated by the other scales in the subgroup analysis. In the patients reporting an improvement in their condition, large effect sizes reflected changes in the expected direction on the Chalder Fatigue Scale, especially on the physical fatigue subscale, the timed walk, the Social Functioning subscale of the SF-36 and SF-36 Pain. Moderate effect sizes were demonstrated on

the Barthel, the 'Locking' VAS, Chalder's Mental Fatigue scale, SF-36 Physical Functioning and SF-36 Change in Health. Small effect sizes were found on the Pain VAS, ABES Body Totality, timed stands and SF-36 Role Physical, Energy/Vitality and General Health Perceptions.

In those patients who judged their condition to have stayed the same, patients showed a small improvement on Chalder's Fatigue Scale and a small deterioration on both the Energy/vitality and General Health Perceptions scales of the SF-36.

For patients reporting their condition to have deteriorated, small discrepant improvements were demonstrated on the Pain VAS, Chalder's Physical Fatigue subscale, and the PGI. A moderate effect size demonstrated a discrepant improvement on the 'Locking' VAS. Scores on the SF-36 demonstrated the expected deterioration in score on all the subscales except for Role Emotional, which reflected a small improvement.

Table 11.30: Effect sizes in validated health status measures by subgroups representing patients who reported either an improvement, a deterioration or no change in their condition

Measure	Effect size in patients reporting an improvement in their condition (4 patients)		Effect size in patients reporting staying the same (14 patients)		Effect size in patients reporting an deterioration in their condition (7 patients)	
	Mean change	Effect size	Mean change	Effect size	Mean change	Effect size
Barthel	**1.25** *(I)*	**0.5**	-0.07 *(D)*	-0.019	0.14*(I)*	0.068
Weakness VAS	-2.5 *(I)*	-0.138	3.85*(D)*	0.126	-2.43*(I)*	-0.010
Pain VAS	**-6.75** *(I)*	**-0.240**	-1.85*(I)*	-0.058	**-5.85***(I)*	**-0.375**
'Locking' VAS	**-8.25** *(I)*	**-0.508**	-3.36*(I)*	-0.130	**-14***(I)*	**-0.896**
Fatigue score	**-4.75** *(I)*	**-0.927**	**-1.64***(I)*	**-0.318**	-0.57*(I)*	-0.097
Physical fatigue	**-4.5** *(I)*	**-1.071**	**-1.29***(I)*	**-0.284**	**0.14***(D)*	**0.340**
Mental fatigue	**-2.75** *(I)*	**-0.601**	**-0.79***(I)*	**-0.376**	**-0.71***(I)*	**-0.373**
Patient Generated Index	-2.9 *(D)*	-0.092	3.195*(I)*	0.124	**6.299***(I)*	**0.270**
ABESr (Body totality)	**1.75** *(I)*	**0.294**	-0.5*(D)*	-0.073	1.86*(I)*	0.177
ABESr (Body Self-consciousness)	-2.25 *(D)*	-0.180	-2.08*(D)*	-0.197	0.28*(I)*	-0.035
Timed walk	**-1.258** *(I)*	**-1.17**	0.708*(D)*	0.104	**-0.329***(I)*	**-0.021**
Timed stands	**-1.17** *(I)*	**-0.442**	0.814*(D)*	0.05	**-3.001***(I)*	**-0.080**
SF36 Physical functioning	**12.5** *(I)*	**0.66**	2.5*(I)*	0.063	**-10** *(D)*	**-0.388**
SF36 Role Physical	**25** *(I)*	**0.433**	-7.14*(D)*	-0.173	**-14.28**	**-0.378**
SF36 Role emotional	8.25 *(I)*	0.165	2.41*(I)*	0.054	**9.57***(I)*	**0.300**
SF36 Social Functioning	**32.25** *(I)*	**0.867**	1.09*(I)*	0.036	-3*(D)*	-0.120
SF36 Mental Health	0.25 *(I)*	0.009	-1.14*(D)*	-0.08	-1.14*(D)*	-0.075
SF36 Energy/vitality	**12.5** *(I)*	**0.333**	-4.64*(D)*	**-0.205**	**-10***(D)*	**-0.800**
SF36 Pain	**33.25** *(I)*	**1.123**	0.05*(I)*	0.002	**-15.94***(D)*	**-0.306**
SF36 General Health Perceptions	**10** *(I)*	**0.419**	**-4.14***(D)*	**-0.226**	**-6.86***(D)*	**-0.298**
SF36 Change in health	**18.75** *(I)*	**0.786**	-1.79*(D)*	-0.066	**-5.86***(D)*	**-0.298**

† Bold print indicates effect size of note, *I*= Improvement, *D*= Decline

11.2 Discussion

The important properties of health status measures include reliability, validity, interpretability and responsiveness to clinically important change (Feinstein, 1987) (McDowell and Newell, 1996; Streiner and Norman 1995; Fitzpatrick et al, 1998; Hays and Hadhorn 1992). In order to ensure that these properties were fulfilled, a series of steps were taken to evaluate the new questionnaire. The results of this evaluation will be discussed in the following pages.

11.2.1 Interpretability

The wide distribution of scores on the various subscales demonstrates the ability of the measure to pick up a broad range of symptom and disease impact and therefore also its potential to detect change. The normal distribution of NMD-related QoL scores further suggests that the scale is likely to be responsive to change.

Roughly equal numbers of respondents obtained scores across the range possible for Weakness and Fatigue, indicating the ability of the scale to represent patients at varying levels of symptom impact. As expected, a large number of respondents reported having no muscle 'locking' and a considerable number did not have any pain. This accounts for the skewed distribution of domain scores on these dimensions. However, in the patients who did report an impact of these symptoms, scores were evenly distributed from the lowest to the highest scores possible.

Scores on the Activities, Emotions and Body Image dimensions were evenly dispersed across the spectrum. Independence was slightly skewed towards the lower end of the scale. This is likely to be because a number of patients in the study experienced only mild NMD symptoms, and they were therefore able to maintain a good level of

independence. The Relationships dimension was particularly skewed towards the lower end of the scale, although responses still ranged from the lowest to the highest score possible. This floor effect suggests that the scale may allow little room for improvement. However, it may be that many NMD patients' relationships fare well despite the effects of their condition. This is supported by findings about the positive influence of social support on coping with illness (Sarason et al, 1985) and those showing illness to have a positive effect on relationships (Padilla et al, 1990).

The pattern of differences in scores across the different disease subgroups indicates the ability of the questionnaire to reflect differing amounts of disease impact. For example, most of the subscale scores were higher for patients diagnosed with an ASP condition. The higher scores elicited in this group may be due to the small sample size, greater disability experienced in these patients, their older age and the later onset of their condition. Not only may they have had less time to adapt to their condition, they may also have less social support.

The high levels of weakness reported by patients in the ASP group are likely due to the fact that inclusion body myositis patients commonly receive their diagnosis following problems with weakness. This symptom is particularly characteristic of these patients whereas patients with an acquired, relapsing, remitting condition may go through periods of remission where they experience little weakness. Similarly, patients with a congenital condition may have a diagnosed muscle condition but experience little noticeable weakness.

It was to be expected that only a small proportion of respondents would report muscle 'locking'. This is because the symptom of myotonia or muscle 'locking' is

experienced by patients with myotonic dystrophy or myotonia congenita and is not a feature of the other muscle conditions. This explains why none of the ASP patients reported this symptom and the low mean and median score of patients in the ARR group. However, the high maximum score of muscle 'locking' in the AAR group indicates that this particular question may need further refinement to ensure comprehension. The maximum score on this symptom scale for the CSP group was still relatively low, suggesting that patients may not feel that symptom is considerably bothersome compared, for example, to the symptom of muscle weakness.

Pain is less of a feature in muscle conditions than muscle weakness, which explains the low pain scores on the INQoL. The median score of zero for congenital patients indicates that a considerable number of these patients do not experience pain.

Despite these findings it is still important to include 'locking' and pain in the questionnaire as they are important features of NMD for many patients that influence QoL and can be amenable to treatment.

The ASP group in particular had high scores for negative impact upon their activities, which is likely also due to their older average age and the associated limitations. The congenital group had a lower median score for Activities which may be because a number of these patients were only mildly affected by their condition. These diagnoses are often made following the diagnosis of another family member, not always as a consequence of intrusive physical symptoms.

Although there was wide variation in scores on the Emotions subscale, the scores obtained by the ASP group did not demonstrate such wide variability, which again is likely due to the small sample size.

Once the INQoL has been used more extensively the significance of particular profile scores will become clearer.

11.2.2 Validity

The results of the validation study demonstrate the INQoL's acceptability to patients and ease of completion. The profile of scores also provides useful information about patients' symptoms and NMD impact upon important areas of life.

11.2.3 Face and Content Validity

The use of qualitative data detailing NMD patients' experiences of muscle disease as the basis for the INQoL, maximised the face and content validity of the scale. These properties were confirmed during pilot testing of the instrument (section 8.6, p.196).

11.2.4 Construct Validity

The lack of consensus on a definition of QoL means that it is difficult to establish the validity of QoL scales. There are no existing gold standards of QoL, therefore validity was established by investigating relationships between the dimensions of the INQoL and a series of validated scales hypothesised to measure constructs associated with NMD-related QoL.

The relationship demonstrated between the subscales of the INQoL and the established measures confirmed many of the hypotheses. Correlation coefficients ranged between 0.29 to 0.78 with many greater than 0.5, indicating that the reference measures are related to the subscales of the INQoL but measure a different entity. Correlations of this magnitude were to be expected considering the differences

between the INQoL and the established scales in their scaling methods, underlying constructs and development for use in different circumstances.

11.2.4.1 Functional ability and QoL dimensions

Patients with more functional disability are likely to experience greater difficulty in performing physical activities and more likely to need help in performing many of these activities. Therefore, it was predicted that the greater the patient's functional disability, the higher their scores would be on the dimensions of Activities and Independence. This hypothesis was fulfilled with the timed stands test demonstrating a strong relationship with Activities (Rho= 0.76, 0=0.000) and Independence (Rho= 0.82, p= 0.000). The ten metre walk also demonstrated significant but slightly more modest correlation coefficients of 0.45 (p=0.006) and 0.67 (p=0.000) with Activities and Independence respectively. The stronger relationship between these dimensions and the timed stands test than they have with the ten metre walk supports their validity. This task takes longer, is more physically demanding and therefore more reflective of physical disability.

11.2.4.2 Functional ability and INQoL Symptoms

Of the symptoms, weakness demonstrated a strong and significant relationship with both timed tasks with a Spearman's Rho of 0.50 (p=0.000) for the timed walk and 0.77 (p=0.000) for the stands. Neither Fatigue nor Muscle 'locking' demonstrated a significant relationship with these tasks. Pain had a significant but weak correlation with the timed stands test in the subgroup analysis of patients reporting pain.

These findings support the validity of the symptom scores given the considerable impact that weakness has upon physical tasks and the influence that pain is likely to have in slowing down task performance.

The lack of relationship between the 'locking' and fatigue scores and the timed tasks indicates that these symptoms have a less important role in short term physical activities. Muscle 'locking' (or myotonia) may play a role in the performance of certain activities. However 'locking' as reported by patients on the questionnaire did not seem to influence time taken on these tasks. Similarly, the fatigue reported by patients is unlikely to have had a considerable effect on short-term physical activity. The kind of fatigue associated with muscle conditions is more likely to influence physical activity over longer periods of time.

It would have been interesting to include a test of upper limb function as well as the timed walk and stands task. Measures that can be used for this purpose and that could be adopted in further validation of the INQoL include dynamometry to determine grip strength and peg tests to measure arm disability (Wade, 1992). Peg tests involve timing patients' performance in placing a set number of pegs into holes. They can be sensitive, however they focus on manual dexterity rather than arm strength and they are not sensitive in measuring degrees of moderate to severe disability.

11.2.4.3 Symptoms

Symptom scores on the new scale and those on the established measures were strong. Spearman's Rho coefficients ranged between 0.69 and 0.78 for the weakness, 'locking' and pain scores of the INQoL and respective VAS scales. The fatigue subscale demonstrated a slightly more modest relationship with Chalder questionnaire

scores (Rho= 0.49, p= 0.000). These strong relationships were to be expected given that physical symptoms are less likely to be influenced by the individual's situation or their subjective interpretation than ratings of disease impact on life dimensions. However, symptom impact as well as symptom severity is measured in the INQoL, placing the effect of symptoms within the context of the patient's life and their adaptation to symptom effects. The VASs measure symptom severity alone, which explains why the correlation between these measures was not even stronger. The Pain scale of the SF-36 includes a question about the interference of pain as well as its severity, which accounts for the strong relationship between this and the Pain scale of the INQoL (rho= -0.78, p=0.000).

Weaker concordance between scores on the fatigue subscale and those on Chalder's fatigue scale is likely to be due to the emphasis of Chalder's scale on a change in fatigue over the last 6 weeks, rather than upon fatigue experienced 'at the moment' as measured by the INQoL.

11.2.4.4 Activities

The Physical functioning subscale of the SF-36 measures limitation in performing daily activities such as lifting, walking, climbing stairs and self-care. The activities dimension of the INQoL measures the impact of NMD upon daily activities, leisure activities and work. It was therefore predicted that these two scales would correlate with each other. The hypothesis underlying this premise was that patients who experience greater difficulty in physical activities and self-care tasks would report more problems in carrying out activities and greater dissatisfaction with their ability to complete these activities. This was supported by the correlation between the scores on the Physical Functioning scale of the SF-36 and the Activities dimension of the

INQoL (Rho= -0.59, p=0.000). Unlike the INQoL, the SF-36 focuses on activities of daily living. It does not specifically ask about work activities, and leisure activities are only implied in items about vigorous and moderate activities. This may account for the more moderate correlation between these scales than might have been expected.

11.2.4.5 Independence

The Barthel scale assesses functional ability and degree of independence in carrying out activities of daily living, such as dressing, grooming and bathing. The independence dimension of the INQoL measures NMD impact upon patients' independence and satisfaction with their level of independence. The correlation between these scores (rho= 0.67, p= 0.000) therefore supports the hypothesis that those with difficulties in mobility and self-care tasks would report lower levels of independence and greater dissatisfaction with their level of independence.

11.2.4.6 Social Relationships

The Social Relationship subscale of the new questionnaire measures the impact of NMD upon relationships with partner/spouse, family, friends and other social contacts. It also looks at patient satisfaction with these social relationships and the importance patients attach to the effects of NMD upon them. The SSQ6 measures social support, in terms of the number of people available to provide support in various circumstances and their satisfaction with this support. The small but significant negative correlation between these scales testifies to their measurement of differing constructs. However, it also indicates that social support plays a role in maintaining positive relationships.

Social Functioning as measured by the SF-36, FLP Social Interaction and NHP Social Isolation yielded much stronger associations with the Social Relationships dimension of the INQoL. This is likely to be because they measure the impact of illness upon the dynamics of social relationships, whereas social support, is more important in mediating the relationship between life events and illness (Sarason et al, 1985). The moderate correlation coefficients demonstrated between the social support scores and NMD-related-QoL as measured by the INQoL supports the hypothesis that social support plays a role in maintaining QoL.

11.2.4.7 Emotions

The Emotions dimension of the INQoL measures the impact of NMD upon the emotional well-being of the respondent, specifically whether it causes the patient to feel depressed, anxious, low in confidence or frustrated by their condition. It also measures patients' satisfaction with their emotional well-being. The HAD scale screens for depression and anxiety and as expected, its subscales were strongly related to the Emotions dimension of the INQoL (HADS Anxiety; Rho= 0.63, p=0.000) (HADS Depression; Rho= 0.66, p=0.000).

The Emotions subscale of the FLP includes nine items relating to depression, anxiety, irritability and emotional stability. The Emotional Reactions subscale of the NHP includes nine items, which cover feelings of anxiety, depression and loss of control. Correlation of INQoL Emotions subscale with these subscales further supports the validity of the dimension. The closely related construct, Mental Health as measured by the SF-36 was also significantly correlated to the Emotions subscale of the INQoL.

11.2.4.8 Body Image

The ABES measures the impact of arthritis upon body image, more specifically how patients feel about their physical appearance, the way their body moves and how self-conscious they are about their body. The Body Image subscale of the INQoL measures the perceived impact of NMD upon physical appearance and upon patients' satisfaction with the way they look.

The strong relationship between the subscale scores of the ABES and the INQoL subscale support the validity of the scale in measuring body image. INQoL Body Image and Body Totality on the ABES demonstrated a stronger correlation than the relationship between INQoL Body Image and Body Self-consciousness. This was to be expected as the Body Totality scale measures impact upon satisfaction with appearance rather than embarrassment due to physical effects of their condition, which is a rather more specific construct.

11.2.4.9 Quality of Life

The composite QoL score of the INQoL is generated by combining the scores representing patients' satisfaction with each of the five dimensions with the importance attached to NMD impact on these dimensions.

The PGI measures the impact of disease upon the five most important life areas affected by the patient's condition, 'all other health-related aspects of life' and 'all non-health aspects of life.'

The significant relationship between these measures supports the validity of the INQoL composite score. The PGI purports to provide a score that represents

individualised health-related quality of life and, like the INQoL is based on the definition of QoL as the difference between an individual's current state and their expectations or ideals (Calman, 1984). The considerable overlap between the dimension of the INQoL and domains generated by patients on the PGI further supports the content validity of the scale (Table 11.24) and accounts for the relationship between the QoL scores on these two scales. However, as the PGI requires patients to generate dimensions of individual relevance it was inevitable that a number of the domains generated would differ from those on the INQoL. This and the incorporation of global ratings of other aspects of health and life in general accounts for the slightly weaker relationship between the PGI and QoL as measured by the INQoL than might have been expected.

The FLP can be aggregated into a single index and covers a broad range of physical and psychological areas of life important to QoL in individual patients with physically disabling conditions. There was a strong relationship between the FLP and the QoL score on the INQoL, further supporting the validity of the new scale.

To sum up, the association of INQoL dimensions with scores on related measures supported the hypothesised underlying constructs of the scale. This demonstrates the construct validity of the INQoL as a measure of QoL in patients with NMD.

The INQoL's association with the FLP, a measure of disease impact in disabling conditions and the PGI, an individualised measure of health-related QoL, convincingly supports the validity of the INQoL. From this it may also be concluded that the FLP and PGI provide useful measures of QoL for NMD. However, there are limitations to the use of both the FLP and the PGI that are likely to impede their

uptake. For example, the FLP is long and takes an average of 20 minutes to complete. It also has a complicated scoring system that involves the aggregation of individual item weights. Furthermore, a large number of FLP items do not apply to a considerable number of patients. This is likely to dampen respondents' motivation to respond accurately and to complete the assessment.

The PGI involves the generation of individually relevant items and is therefore likely to result in an accurate picture of individual patients' QoL. However, it is not suitable for use in many settings due to problems incurred in self-completion. The generation of individual domains and the complicated system of item weighting through point spending has resulted in low response rates (Macduff and Russell, 1998). The item generation phase may also mean that important issues are missed if they do not come to mind when completing the scale. It is therefore best if a trained interviewer administers the PGI. However, this is more time and resource consuming and means that the scale is not practical for use in most clinical and research settings.

The SF-36 has also been used in muscle disease, more specifically in studies of myositis (e.g. Alexanderson, 1999). Many of the SF-36 dimensions demonstrated strong relationships with the INQoL scales (e.g. Pain and SF-36 Pain, Activities and SF-36 Physical Functioning, Relationships and SF-36 Social Functioning) which suggests that these subscales may be useful in capturing aspects of QoL in NMD. However, as with the FLP, a number of SF-36 items are not relevant to many patients with NMD. For example, some of the items refer to functional activities that involve walking and climbing stairs, which are not applicable to wheelchair users. These are therefore inappropriate as well as limited in their ability to detect change in many patients. The generic nature of the scale also means that many items are not specifically relevant to the

impact of NMD, for example, the item 'have you been a happy person' could be influenced by many different factors. Similarly, the relevance of questions such as 'seen I get ill more easily than other people' (Qun 11a) provide little information about the impact of NMD on QoL.

The INQoL therefore provides a more relevant and practical measure of QoL in NMD than these generic measures of QoL. It allows respondents to rate the impact of specific symptoms, the influence of NMD upon life areas, incorporates ratings of individual importance and can be completed without the assistance of an interviewer.

11.2.5 Reliability

The reliability of an instrument is the degree of consistency with which it measures the attribute of interest. This can be influenced by external sources, such as the individual respondents or the observers who score the measure. It can also be influenced by sources internal to the measure such as scaling or question wording. The INQoL is a multidimensional instrument, and it was therefore considered inappropriate to measure its internal consistency as items in the separate subscales were designed to measure different aspects of QoL.

However, as it is important that the scale is able to detect real change, the test-retest reliability of the scale was investigated. This was done by measuring agreement between the scores obtained on two administrations of the scale separated by one week. This was considered short enough to minimise the chance of any change in the patients' condition but long enough to ensure that previous responses would not be recollected.

The Weakness and 'Locking' scales demonstrated good levels of agreement between scores on these scales at time one and time two. Pain and Fatigue scores demonstrated slightly lower levels of agreement, but this may have been due to a real change in these symptoms between the two time points. Both pain and fatigue are likely to vary considerably over short periods of time and it may be that the questionnaire reflected changes in these symptoms between test and retest.

Consistency was demonstrated between scores obtained on the first and second completion of the scale in the Activities, Independence, Social Relationships and Emotions subscales. Body Image scores were less consistent, but demonstrated an acceptable level of agreement.

Seven of the ten subscales demonstrated a mean score that was slightly lower on retest, suggesting that there may have been some kind of systematic bias between the administration points. More patients completed the scale at home on their second completion of the scale. It could be that patients felt better when completing the scale at home, having not had the journey to the hospital or anxiety associated with attending a research appointment in a hospital setting. It could also be that respondents were more inclined to give negative responses when completing the measure in the presence of a researcher (investigator expectancy bias).

Variability in scores from initial test to retest is likely to be due in part to the small sample size. If a larger sample size had been adopted the variance is likely to have been smaller as a greater number of difference scores would be likely to fall around the mean. Nonetheless, seven of the ten profile scores demonstrated limits of agreement that were less than 35 points from zero (point of no change). Greater

variability was demonstrated in the change in scores between times one and two in the Pain, Fatigue and Body Image subscales.

The individualised nature of the questionnaire may also have influenced its reliability. Mid-range responses are more likely to be used with standardised scales (Lacasse et al, 1999) whereas importance ratings may elicit more polarised responses at the extreme ends of the scale.

It is likely that the agreement between scores at test and retest would have been better if the conditions had remained the same at both time points. For those questionnaires completed at home it is uncertain whether the whole questionnaire was completed in one sitting and whether it was completed by the patient or by someone else. Furthermore, for respondents' completing the scale at home on retest there was little control over when the scale would be completed. This resulted in many respondents completing the scale more that one week after the initial test.

There are a number of steps that could be taken to improve the appearance of test-retest reliability. For example, the use of a larger sample would reduce the variance in the difference between scores at time one and time two. Shortening the period between test and retest and having respondents complete the scale under the same conditions on both occasions would also improve the appearance of reliability.

11.2.6 Responsiveness

The ability to detect clinically important change is an essential property of clinical tools designed to monitor patients' progress over time or detect change in response to treatment (Guyatt et al, 1987; Fitzpatrick et al, 1992). Due to the time scale of the

project and difficulty in recruiting patients receiving treatment, a preliminary rather than complete assessment of responsiveness was undertaken.

Most of the changes reflected by the scales used in the study were small. However, even in the parameters demonstrating a substantial change, the sample size was too small for the effect size statistics to be conclusive.

When changes in the group as a whole were analysed, the Pain and Emotions scales demonstrated small effect sizes. These were not mirrored by changes in the reference measures. However, when patients were divided into groups representing their perception of whether they had improved, deteriorated or stayed the same, changes in the expected direction indicated the INQoL's potential to respond to change. For example, the small effect size on the Fatigue dimension in patients who had 'improved' was mirrored by substantial improvements on the Chalder Fatigue scale. Faster times on the timed tasks in those who reported an improvement supported corresponding effect sizes in these patients for the Activities and Independence dimensions.

In the patients reporting improvement in their condition, the small effect size on the Activities subscale was also reflected in the Physical Functioning subscale of the SF-36. The small improvement on the Independence subscale was also echoed by a moderate effect size on the Barthel index. However, improvement in Emotions as measured by the INQoL in this subgroup was not mirrored by an improvement on the Mental Health subscale of the SF-36. This might be because the scales measure somewhat different constructs. The INQoL measures the emotional impact of NMD whereas the SF-36 asks questions relating to general mental health (e.g. 'have you

been a very nervous person', 'have you been a happy person').

Similarly, the small effect size indicating an improvement in NMD-related QoL in patients reporting an improvement was not reflected by the PGI. This might suggest that the PGI does not adequately represent change, particularly considering the improvement in PGI scores in patients who reported a decline in their condition.

The only notable change of score in patients reporting a decline in their condition was on the Pain subscale. This decline was not mirrored in the Pain VAS, although a small increase in Pain was registered on the SF-36. There were discrepant improvements in patients reporting a decline in their condition on numerous scales including the 'Locking' VAS, the PGI and the timed functional tasks. This suggests that the condition of these patients may not have deteriorated. However, the increase in pain as reported on the INQoL may account for the reported decline in condition. This reported deterioration may also be due to their diagnosis. Six of the seven patients who reported a decline in their condition had a congenital, slowly progressive condition and one had an acquired, slowly progressive condition. It may be that these patients expected to have become a little worse since their initial visit, even though there may have been no notable change.

Patients in the group that reported an improvement in their condition are more likely to have experienced a real change as three of these four patients had a relapsing remitting condition and may have gone into remission as the result of steroid treatment.

These results indicate the potential of the INQoL to be responsive to change. However, a study involving a larger sample of patients would be necessary in order to draw a more conclusive estimate of responsiveness. Ideally responsiveness should also be assessed in patients undergoing an intervention known to have a significant clinical effect. This is difficult in NMD as there are few treatments that have a proven beneficial effect. A rigorous test of responsiveness would therefore require a multicentre effort in order to recruit a large enough sample in receipt of such a treatment.

The responsiveness demonstrated by the various measures may also have been better had patients been split into groups according to reported change in QoL rather than change in their condition. As discussed in chapter one, individuals tend to respond in different ways to the physical effects of illness and QoL levels may not always reflect physical or functional state. Therefore, changes demonstrated by patients subdivided into groups according to a reported change in QoL are likely to demonstrate greater correspondence to changes in QoL measures and other scales.

11.3 Conclusion

The INQoL has demonstrated good validity and reasonable levels of reproducibility and provides a promising measure of QoL in NMD. A preliminary study of responsiveness indicating the potential of the INQoL to be responsive to change indicates its usefulness as an outcome measure for clinical research. These properties also make the INQoL attractive as a clinical tool that could be used to document changes in QoL between consultations.

CHAPTER XII

THE CLINICAL UTILITY OF THE INQOL

Chapter 12: The Clinical Utility of the INQoL

12.1 Introduction

Clinical instruments that look at quality of life can be useful in monitoring patients' condition between appointments and have the potential to highlight issues that may be overlooked in a brief clinic appointment (Fitzpatick et al, 1993).

In order to see whether the questionnaire would be used during the consultation and whether it would highlight particular issues or influence doctor-patient communication, a small observational study was conducted. This pilot of the INQoL as a clinical tool also enabled testing for feasibility in terms of time and ease of administration.

12.2 Methods

The practicality of using the INQoL as a clinical tool in routine clinical practice was assessed in the outpatient muscle clinics of two consultant neurologists in a small pilot study. Patients due to attend King's College Hospital for a consultation were contacted and invited to attend the clinic half an hour before their appointment in order to complete the questionnaire. The questionnaire was administered to 6 patients before their consultation. It was then scored and a graphical representation of the patients' profile of scores including overall QoL was passed onto the doctor at the beginning of the patients' consultation (see Appendix G).

The consultation was tape recorded in order to capture all the issues discussed and to see whether the questionnaire influenced the consultation.

Patients were also briefly interviewed following their appointment. Specifically, they were asked whether they felt the questionnaire had influenced the consultation, in what way it had been influenced and how they felt the assessment fitted into their clinic visit.

In order to determine ease of scoring and lucidity of the scoring scheme, the INQoL was also scored by a Consultant Neurologist and by a research assistant, both previously unfamiliar with the scoring system.

12.3 Results

12.3.1 Patients

Six patients took part (5 M and 1 F). Four had a congenital NMD (e.g. muscular dystrophy) and two had inclusion-body myositis.

12.3.2 Tape recorded consultations

The INQoL profile was discussed in all six of the consultations, which varied considerably across the different patients depending on factors such as whether they were receiving treatment, whether they were working and their family situation.

Issues of particular relevance to QoL that were captured in the questionnaire but were still either elicited by the consultant or brought up independently by the patient included practical problems such as difficulties in maintaining working activities. This was discussed in two of the consultations. Problems with transport (both public transport and driving) were discussed in three of the consultations. One patient mentioned fatigue as a significant factor in their decision to cut down on work.

The INQoL triggered discussion about psychosocial issues including confidence in going out and pursuing leisure activities, about independence and about social relationships.

Wheelchairs and walking aids were discussed in two of the consultations and the INQoL was referred to in relation to the impact that this kind of intervention could have in terms of activities and independence.

During the consultation one of the neurologists commented that symptom scores gave a good reflection of the individual clinical cases. Life domain scores and factors relating to symptoms and their impact on particular life domains were also discussed. It was remarked that the profile of scores would be of particular interest once the patient returned for a follow-up appointment and their INQoL profile compared with that of their last consultation. This would allow an evaluation of change in response to intervention and advice or as a result of any progression in the patient's muscle condition.

12.3.3 Patients' comments following the consultation

Patients believed that the questionnaire influenced the consultation positively in that it prompted them to mention concerns and issues not previously raised. They also liked that the questionnaire highlighted broader issues other than their physical well-being (e.g. emotional well-being and independence). One respondent mentioned that he would have liked to have been asked about more specific functional activities, such as staircases and obstacles.

The time taken to complete the measure before the consultation was believed to be acceptable.

12.3.4 Scoring the INQoL

The questionnaire took five minutes to score by hand. This was considered to be somewhat lengthy for the questionnaire to feasibly be incorporated into a busy clinic, unless there were someone available to score the questionnaire before the consultation. This would most likely require a member of staff employed specifically to administer and score the forms.

As a result of this an Access database was developed to automatically score the questionnaire. With this database it takes approximately one minute to score each form. This should facilitate the incorporation of the scale into muscle clinics as the questionnaire could be easily scored and a printout of the patient's profile provided for use during the consultation and to be filed in patient notes for comparison with scores on follow-up consultations.

12.4 Discussion

The INQoL is the first QoL measure designed specifically to measure QoL in NMD. In the pilot study it was shown to represents individual patients' QoL and encourage discussion about the impact of symptoms upon broader areas of life and ways of addressing difficulties.

Questions about more functional activities, as requested by one of the patients are best covered by disability scales such as the HAQ (Fries et al, 1980).

In other specialities such as rheumatology (Guillemin, 2000) and oncology (Velikova et al, 1999) there has been increasing use of health status measures (including the

HAQ) in clinical practice. These measures are useful for indicating the impact of conditions like rheumatoid arthritis in which disease impact varies considerably and for which the treatment has varying degrees of effectiveness. Disability scales and QoL measures may also be useful in guiding clinical care and management in NMD given the progressive nature of these conditions, their disabling effects and the paucity of effective treatment.

The clinical utility of questionnaires is hard to capture accurately and the actual practicality of QoL questionnaires in the UK neurology setting is uncertain. At the moment it is uncommon for outcome measures to be used in the clinic and therefore there are no resources and structures (e.g. staff, technology) available to support their use.

The INQoL can be scored by hand which requires clinic staff to familiarise themselves with the scoring system. For the Access database scoring version the responses can be entered into a database and then the programme can score the questionnaire automatically. This requires the appropriate computer equipment and software and for there to be someone available to enter the data. Resources and facilities must therefore be in place before such questionnaires become commonplace in clinical consultations. Ultimately, there may be some kind of computerised interactive questionnaire that could be filled in directly by patients and scored automatically. This would result in a graphical representation of the QoL profile available to the doctor at the same visit that would facilitate comparisons across successive consultations.

12.4.1 Future work on clinical utility

There is a need to test the utility of the INQoL in a larger study involving different outpatient departments to see whether it would fit into these differing systems and to assess the generalisability of the scale. This should be done over the period of at least a year to indicate whether QoL information benefits clinical care and whether changes in INQoL score provide useful information about the patient's condition and their adaptation over time.

CHAPTER XIII

DISCUSSIONS

Chapter 13: Discussions

This project aimed to develop and validate a QoL questionnaire specific to NMD that would represent the impact of NMD in the context of individual patients' lives.

Muscle diseases are much less common than chronic conditions such as arthritis, diabetes and asthma, for which there have been a proliferation of health status measures. However, the chronic and progressive nature of these disabling conditions makes quality of life a major goal in their treatment. The INQoL was therefore created to fill the need for a muscle disease-specific outcome measure that could be used in research and as a clinical tool.

The clinimetric properties of the scale have been established through a rigorous validation process. This means that the measure can confidently be used alongside biological and functional measures to determine the effectiveness of treatment from the patients' perspective.

The INQoL consists of 45 questions within 10 sections. Four of these focus on the impact of key muscle disease symptoms, five look at the impact of NMD on particular areas of life and one section asks about the effects of treatment. This results in a profile of ten scores, nine of which represent the sections of the INQoL and a composite score, based on scores from the five life domain.

Two other scores can be derived from the treatment section. These represent patients' perceived treatment effects and expected treatment effects respectively.

13.1 Questionnaire development

Semi-structured qualitative interviews allowed the investigation of all the areas of life that could potentially be influenced by NMD. This was followed by a postal survey that required patients to rate the degree to which particular areas of their lives are influenced by NMD. This verified the domains of life that were important to include in the questionnaire. Results from this survey enabled items to be selected on the basis of their importance to patients, thereby maximising the likelihood that the measure would accurately capture QoL.

13.2 Implications of the study

The INQoL represents individual patients' quality of life making it a useful measure of change in perceived symptom impact and also in specific areas of life.

The INQoL has already demonstrated good construct validity but the clinimetric evaluation of the scale should continue as the questionnaire goes on to be used in different areas of research. This will further increase confidence in the significance of scores generated by the INQoL.

The INQoL can also be used as a clinical tool that could be used to highlight individual concerns and help monitor patients' progress and the effectiveness of intervention over time.

Over time the way in which different interventions influence individual scores on the measure will become apparent. It is likely that physical, social and psychological interventions will each influence scores on the INQoL differently. For example, independence may improve following the acquisition of an electric wheelchair, and

psychological interventions (e.g. coping skills training, cognitive behavioural therapy) could improve emotional well-being, body image or social relationships.

13.3 Limitations

The INQoL demonstrated a good level of test-retest reliability. However, this may have been higher if not for some limitations in this part of the study. For example, the sample size was quite small, and a considerable number of respondents completed the scale at home on their second completion due to long distances involved in travelling to study appointments. If conditions had been more similar between test and retest, with a greater degree of control over the period between the two time points agreement between the scores is likely to have been better.

It was also difficult to obtain a good idea of how responsive the scale is to change. Few patients receive treatment, and in those who do, there is often uncertainty about how effective the treatment will be. In order to obtain a good idea of questionnaire responsiveness a large sample of patients should be studied over a prolonged period during which they are likely to demonstrate change.

13.4 The way forward for the INQoL

13.4.1 INQoL responsiveness

Further work is needed to establish the responsiveness of the INQoL to clinically important change. Given the comparative rarity of NMD and the lack of effective treatment, the most appropriate forum for the investigation of scale responsiveness would be a large scale multicentre clinical trial, comparing reported change in QoL and traditional outcome measures (muscle strength, functional measures) with change on the INQoL.

As there were no large scale clinical trials taking place during the timescale of the project it was not possible to conduct a thorough investigation of responsiveness. However, there are plans to continue the validation of the INQoL in upcoming trials. These studies will also provide opportunities for cross-cultural validation of the INQoL (see sections 13.4.3 and 13.4.4).

13.4.2 Module Questions

Question two, which asks about 'muscle locking' is one of the symptom questions that could be treated as an 'add in' or 'leave out' question, depending on the population of interest in a particular study. This question only has relevance for a small number of patients (specifically those patients with myotonia congenita or myotonic dystrophy). Its inclusion in the questionnaire is superfluous for many patients and may be confusing for those who do not experience this symptom. By turning this question into a module item, the responsiveness of the questionnaire might be enhanced.

Similarly, the questionnaire could be adapted for use in other diseases by incorporating different symptom questions relevant to the particular patient group of interest.

13.4.3 Using INQoL in other chronic diseases

The sound theoretical grounding and incorporation of importance ratings in the INQoL makes it likely that it could successfully be adapted for use in other chronic conditions. The symptom scales could be altered and the five major life domains maintained, as it is likely that these issues are influenced in numerous other chronic disabling conditions. There are already plans to extend the use of the INQoL to other neuromuscular conditions (myasthenia gravis and periodic paralysis). Extending its

use will involve revalidating the questionnaire to ensure the validity of the dimensions and any individual symptom questions in these particular patient groups.

13.4.4 Cross-cultural validation

There are also plans to adapt the INQoL for use in other countries (specifically Holland, Italy, Germany and the USA). This would involve qualitative studies to investigate issues of importance to patients of other nationalities, which would indicate whether the content of the questionnaire should be adapted. Translation and back-translation of the questionnaire would ensure that adaptation into different languages leads to results that have similar meaning regardless of language (Guillemin et al, 1993; Bullinger et al, 1993). This would enable findings from different countries to be compared or collated for use in large-scale studies.

13.4.5 INQoL Clinical Utility

The pilot study of the INQoL in a clinical setting indicated its usefulness in highlighting individual problems and promoting discussion between doctor and patient about quality of life issues. Its clinical utility should be investigated further in a variety of hospitals to ensure its applicability within different systems. This will increase the weight that can be invested in the significance of INQoL scores in the clinical setting.

As clinicians become more familiar with the scale and the meaning of INQoL scores, its useful application will increase. For example, INQoL scores could be used to support the provision of resources for health care strategies or encourage funding of research into the physical, social and psychological well being of NMD patients.

13.4.6 Other areas of research

13.4.6.1 Proxy measures and carer QoL

There are several other respondent groups in which QoL assessment could be of considerable value.

For example, the measurement of caregiver QoL also deserves greater attention. It has been documented in other conditions including motor neurone disease (MND) that caregivers' QoL can be negatively influenced (Jenkinson et al, 2000; Bromberg and Forshew, 2002).

Individualised measures such as the SEIQoL and the PGI may prove useful in capturing caregiver QoL. Furthermore, measures taken to ameliorate burden may improve QoL for both patient and carer.

13.4.6.2 Children

A QoL measure specifically for use in children with NMD is also lacking. However, the adaptation of the INQoL for children would not be appropriate as the QoL concept differs considerably between children and adults (Rosenbaum et al, 1990). Illness influences the child's development, and the issues of relevance to children differ considerably from those that are important to adults (Hanai, 1996). QoL assessment in children must be therefore be approached in a different way. A thorough investigation of the impact of NMD in children followed by observational studies of the resultant measure's acceptability and appropriateness would have to be conducted alongside validation studies. It is also advisable to develop different measures for different age groups and to adopt different administration methods according to age (e.g. interview vs. self completion) (Christie et al, 1993). Proxies including parents,

schoolteachers and physicians may also provide useful information to complement QoL reports elicited from children (Hoare and Russell, 1995; Finkelstein 1998).

13.4.6.3 The use of technology in QoL assessment

The routine assessment of QoL in research and clinical practice could be facilitated by the use of technology (Guillemin, 2000). Not only can questionnaires be scored using database packages, but questionnaire responses could also be scanned directly into a computer and automatically scored.

Interactive technology including touch-screen programs and internet access to questionnaires may also facilitate the assessment of QoL in the future. Responses to QoL measures could also be registered over the telephone, using the key pad to respond to automated questionnaire items.

The popularity and applicability of these methods remain to be tested. If they prove to be feasible they would facilitate data collection, allowing easy access to greater numbers of patients and for ongoing monitoring of QoL over time.

13.5 The future of QoL assessment

Dramatic developments in health care have led to increased emphasis on improving care for the chronically ill or disabled.

The assessment of QoL in clinic and the implications of this assessment necessitates changes to the clinical consultation as well as the treatment and follow-up of patients. It may also challenge the role of the clinician. Where before the doctor's role has predominantly involved treating physical ailments, the assessment of QoL and the

ultimate goal of improving QoL necessitates addressing broader social and psychological issues.

Some may believe this to be an intrusion into matters that are not the concern of the physician. It would therefore be necessary to conduct studies to determine patients' attitudes towards such changes as well as the practicality and economic feasibility of any change.

Such a shift in healthcare provision would be likely to involve changing from a doctor-centred system to one involving more multidisciplinary teamwork, changes that are already apparent in many healthcare departments (e.g. stroke rehabilitation, diabetes care, addiction services). These teams might involve specialist nurses, occupational therapists, physiotherapists, psychologists and other health professionals working alongside doctors to provide holistic care from a physical, social and psychological perspective.

CHAPTER XIV

CONCLUSIONS

Chapter 14: Conclusions

- Quality of life is a complicated concept that can best be defined as the distance between an individual's current state and the state to which they aspire.

- NMD patients are influenced in all major aspects life, from their activities and independence to their emotional well being.

- NMD symptoms (e.g. weakness and fatigue) underlie many of the problems experienced in these life areas.

- The INQoL was designed based upon patients' experiences of NMD and a theoretical model of QoL. It thereby provides an idea of symptom impact and the influence of NMD upon major areas of life. The INQoL should therefore help to target treatment towards specific needs and in monitoring specific symptoms and their influence upon specific areas of life.

- The Individualised Neuromuscular Disease Quality of Life (INQoL) questionnaire is a valid and reliable measure of QoL in NMD.

- Early indications of INQoL responsiveness should be confirmed in larger studies involving patients in receipt of treatment.

- Early findings indicate the usefulness of the INQoL as a clinical tool that can be used to highlight individual problems and monitor patients' progress. Further work should be conducted to establish its utility in a variety of clinical settings.

- Future studies should include cross-cultural validation of the INQoL and the development of QoL assessments for carers of patients with NMD and for children with NMD.

CHAPTER XV

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CHAPTER XVI

APPENDICES

Appendix A: Coding scheme for qualitative interview study

Physical Symptoms
P- B (Bladder problems)
P- D (Discomfort)
P- F (Fatigue)
P- P (Pain)
P- S (Other symptoms)
P- St (Strength/weakness)

Side Effects.
SE

Medical Procedures.
M

Daily Activities.
DA- E (Eating)
DA- D (Driving)
DA- F (Falling)
DA- G (General)
DA- H (Housework, include cooking)
DA- ML (Mobility-lower body e.g. standing up, walking, climbing stairs)
DA- MU (Mobility- upper body e.g. gripping, lifting))
DA- SC (Self Care e.g. washing, dressing, toileting)
DA- T (Transport)

Leisure Activities.
LA- G (General)
LA- Gar (Gardening)
LA- H (Holidays)
LA- Q (Quiet activities, e.g. reading, knitting)
LA- S (Sport)

Employment(Work)
W- C (Change/adaptation in work)
W- G (General)
W- P (Future propsects)
W- S (Stopping)
W- WP (Working practices)

Financial Matters (Money)
M- W (Related to employment/work)
M- G (General)

Changes in accommodation.
Acc

Emotions.
E- A (Annoyance/irritation)
E- Ang (Anger)
E- Anx (Anxiety)
E- C (Confusion)
E- D (Depression)
E- F (Frustration)
E- G (General)
E- I (Feelings of isolation)
E- LC (Loss of confidence)
E- U (Upset/sadness)

Self-perception
SP- G (General)
SP- P (Physical)

Dependence/Independence
I- D (Desire for more independence/ to maintain independence)
I- L (Feelings of loss)
Dep (Dependence on others)

Future.
Fut- G (General)
Fut- Par (Parenting)
Fut- Prog (Progression)

Planning.
Pl

Social
S- E (Embarrassment)
S- OP (Other peoples perceptions)
S- S (Stigma)

Social Activities
SA-D (Dancing)
SA-Dr (Drinking/pubbing)
SA-E (Eating out)
SA-G (General social outings)
SA-S (Shopping)
SA-V (Visiting friends/family)

Social Relationships
SR- F (Friends)
SR- G (General)
SR- W (Work collegues)

Family

F- A (Family Activities, e.g. outings, visits)
F- E (Emotional upset of family members)
F- P (Parenting)
F- R (Role)
F- Rel (General relationships)

Relationship with Partner/Spouse.

RP- E (Emotional upset of partner/spouse)
RP- G (General)
RP- S (Sex life)

General Effect.

GE
GE- S (Slowed down)

Quality of Life in Adult Neuromuscular Disease.
Postal Questionnaire.

This questionnaire has been designed to assess which areas of your life are affected by your muscle condition, to what extent you are affected and how important these effects are to you.
By understanding these effects, doctors will have a greater understanding of the problems muscle disease causes, enabling them to work towards more effective care and treatment to meet the needs of each individual.

INSTRUCTIONS FOR FILLING IN QUESTIONNAIRE.

There are twelve questions to be completed in this questionnaire. These will require you to place a tick in the box that indicates your response.

EXAMPLE QUESTION.
You do not need to answer this question

a) Is your ability to complete household tasks (e.g. washing up, cleaning, dusting) affected by your muscle condition ?

not at all	slightly	moderately	quite a lot	very much
		✓		

IF IT IS NOT AFFECTED, PLEASE GO TO THE NEXT QUESTION.

b) If your ability to complete household tasks is affected, overall is this effect :

Good ☐ Bad ☑ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition on your ability to complete household tasks?

not at all important	quite important	moderately important	very important	extremely important
	✓			

SECTION A:- ACTIVITIES.

This first group of questions has been designed to assess the impact of your muscle disease on your activities. You will be asked about:

a) Your day to day activities (e.g. washing, dressing, eating, getting around)
b) Your working life (e.g. employment status, working practices, the money you earn)
c) Your social and leisure activities

Question 1

a) Are your daily activities affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF THEY ARE NOT AFFECTED, PLEASE GO TO QUESTION 2

b) If your daily activities **are** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your daily activities?

not at all important	quite important	moderately important	very important	extremely important

Question 2

a) Is your working life affected by your muscle condition? (For example, employment status, working practices, the money you earn)

IF THIS QUESTION DOES NOT APPLY TO YOU (FOR EXAMPLE, IF YOU ARE RETIRED) TICK THIS BOX ☐ AND GO TO QUESTION 3.

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO QUESTION 3

b) If your working life **is** affected, overall is this effect :

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important is this effect to you?

not at all important	quite important	moderately important	very important	extremely important

Question 3

a) Are your social and leisure activities affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF THEY ARE NOT AFFECTED, PLEASE GO TO THE ADDITIONAL COMMENTS SECTION, JUST BEFORE SECTION B

b) If your social and leisure activities **are** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your social and leisure activities?

not at all important	quite important	moderately important	very important	extremely important

Additional Comments

Please use this space if you would like to note down any additional comments about how your muscle condition affects any of your **day to day, work, or social and leisure activities .**

SECTION B:- RELATIONSHIPS AND SOCIAL INTERACTION.

This next group of questions has been designed to find out about the impact of your muscle condition upon your relationships with other people. You will be asked about:

a) **Your relationships with friends**
b) **Your relationships with family members**
c) **Your relationship with your partner/spouse**
d) **Your contact with other people in general (e.g. with strangers, acquaintances and colleagues).**

Question 4

a) Are your relationships with your friends affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF THEY ARE NOT AFFECTED, PLEASE GO TO QUESTION 5

b) If your relationships with your friends **are** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your relationships with your friends?

not at all important	quite important	moderately important	very important	extremely important

Question 5

a) Are your family relationships affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF THEY ARE NOT AFFECTED, PLEASE GO TO QUESTION 6

b) If your family relationships **are** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your family relationships?

not at all important	quite important	moderately important	very important	extremely important

Question 6

a) Is your relationship with your partner/spouse affected by your muscle condition?

IF THIS QUESTION DOES NOT APPLY TO YOU TICK THIS BOX ☐ AND GO TO QUESTION 7.

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO QUESTION 7

b) If your relationship with your partner/spouse is affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your relationship with your partner/spouse?

not at all important	quite important	moderately important	very important	extremely important

Question 7

a) Is your day to day contact with other people (e.g. how you get along with strangers, acquaintances, colleagues) affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO THE COMMENTS SECTION, JUST BEFORE SECTION C.

b) If your day to day contact with other people is affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your day to day contact with other people?

not at all important	quite important	moderately important	very important	extremely important

Additional Comments

Please use this space if you would like to note down any additional comments about how your muscle condition affects any of your **relationships, or your contact with other people in general.**

SECTION C:- PERSONAL FEELINGS.

This next group of questions, starting on the following page has been designed to find out about the impact of your muscle condition upon your personal feelings about yourself and about the future. You will be asked about:

a) Your independence
b) Your emotions
c) Your feelings about your physical appearance
d) Your feelings about the future

Question 8

a) Is your independence affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO QUESTION 9

b) If your independence **is** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your independence?

not at all important	quite important	moderately important	very important	extremely important

Question 9

a) Does your muscle condition affect how you feel emotionally?

not at all	slightly	moderately	quite a lot	very much

IF YOU ARE NOT AFFECTED, PLEASE GO TO QUESTION 10

b) If you **are** affected emotionally, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon how you feel emotionally?

not at all important	quite important	moderately important	very important	extremely important

Question 10

a) Does your muscle condition affect how you feel about the way you look?

not at all	slightly	moderately	quite a lot	very much

IF YOU ARE NOT AFFECTED, PLEASE GO TO QUESTION 11

b) If how you feel about the way you look is affected, is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon how you feel about the way you look?

not at all important	quite important	moderately important	very important	extremely important

Question 11

a) Is your idea of the future affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO THE COMMENTS SECTION, JUST BEFORE SECTION D.

b) If your idea of the future is affected, is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your idea of the future?

not at all important	quite important	moderately important	very important	extremely important

Additional Comments.

Please use this space if you would like to note down any additional comments about how your muscle condition affects **how you feel about yourself in terms of your independence, your emotions, the way you look, or your idea of the future.**

SECTION D:- TREATMENT.

This question is designed to find out about how the treatment you receive for your muscle condition affects your life.

Question 12A

Do you receive treatment (for example,tablets and/or injections) for your muscle condition?

Yes ☐ IF "YES", GO TO QUESTION 12B

No ☐ IF "NO", GO TO QUESTION 12C

Question 12B

a) Does the treatment you receive for your muscle condition have any **good** effects?

none at all	some	moderate	quite a lot	many

IF IT DOES NOT HAVE ANY GOOD EFFECTS, PLEASE GO TO PART (C) OF THIS QUESTION

b) How important to you are the **good** effects of the treatment for your muscle condition?

not at all important	quite important	moderately important	very important	extremely important

a) Does the treatment you receive for your muscle condition have any **bad** effects?

none at all	some	moderate	quite a lot	many

IF IT DOES NOT HAVE ANY BAD EFFECTS, PLEASE GO TO THE "ADDITIONAL COMMENTS" SECTION (TOP OF NEXT PAGE)

b) How important to you are the **bad** effects of the treatment for your muscle condition?

not at all important	quite important	moderately important	very important	extremely important

Additional Comments.

Please use this space if you would like to note down any additional comments about how the treatment for your muscle condition affects your life.

Question 12C.

PLEASE ONLY ANSWER THIS QUESTION IF YOU DO NOT RECEIVE TREATMENT FOR YOUR CONDITION.

a) For what reason **don't** you receive treatment for your muscle condition?

There is no treatment for my muscle condition ☐

The treatment I received in the past did not work ☐

Stopped treatment because of side effects ☐

Other (Please specify_____) ☐

b) How does not receiving treatment for your muscle condition make you feel?

PLEASE WRITE IN THE SPACE BELOW

PLEASE TURN OVER

Section E: Additional Comments.

Please use this space if you would like to note down any other ways in which you feel your muscle condition affects your life, or any additional comments you would like to make.

PLEASE MAKE SURE YOU HAVE COMPLETED ALL PARTS OF THE QUESTIONNAIRE THAT APPLY TO YOU AND THEN FILL IN THE DETAILS BELOW.

Today's Date: ___/____/____

Date of birth: ___/____/____

Sex: Male ☐ Female ☐

What sort of muscle condition do you have (if known) :

_____

How long have you had this muscle condition:

_____

Who do you see about your muscle condition?

A specialist ☐

A GP (General practitioner) ☐

Other (Please specify_____) ☐

Thank you very much for your time.

Appendix C: Postal Questionnaire (final version)

Quality of Life in Adult Neuromuscular Disease. Postal Questionnaire.

This questionnaire has been designed to assess which areas of your life are affected by your muscle condition, to what extent you are affected and how important these effects are to you.
By understanding these effects, doctors will have a greater understanding of the problems muscle disease causes, enabling them to work towards more effective care and treatment to meet the needs of each individual.

INSTRUCTIONS FOR FILLING IN QUESTIONNAIRE.

There are twelve questions to be completed in this questionnaire. These will require you to place a tick in the box that indicates your response.

EXAMPLE QUESTION.
You do not need to answer this question

a) Is your ability to complete household tasks (e.g. washing up, cleaning, dusting) affected by your muscle condition ?

not at all	slightly	moderately	quite a lot	very much
		✓		

IF IT IS NOT AFFECTED, PLEASE GO TO THE NEXT QUESTION.

b) If your ability to complete household tasks is affected, overall is this effect :

Good ☐ Bad ☑ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition on your ability to complete household tasks?

not at all important	quite important	moderately important	very important	extremely important
	✓			

SECTION A:- ACTIVITIES.

This first group of questions has been designed to assess the impact of your muscle disease on your activities. You will be asked about:

a) Your day to day activities (e.g. washing, dressing, eating, getting around)
b) Your working life (e.g. employment status, working practices, the money you earn)
c) Your social and leisure activities

Question 1

a) Are your daily activities affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF THEY ARE NOT AFFECTED, PLEASE GO TO QUESTION 2

b) If your daily activities **are** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your daily activities?

not at all important	quite important	moderately important	very important	extremely important

Question 2

a) Is your working life affected by your muscle condition? (For example, employment status, working practices, the money you earn)

IF THIS QUESTION DOES NOT APPLY TO YOU (FOR EXAMPLE, IF YOU ARE RETIRED) TICK THIS BOX ☐ AND GO TO QUESTION 3.

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO QUESTION 3

b) If your working life **is** affected, overall is this effect :

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important is this effect to you?

not at all important	quite important	moderately important	very important	extremely important

Question 3

a) Are your social and leisure activities affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF THEY ARE **NOT** AFFECTED, PLEASE GO TO THE ADDITIONAL COMMENTS SECTION, JUST BEFORE SECTION B

b) If your social and leisure activities **are** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your social and leisure activities?

not at all important	quite important	moderately important	very important	extremely important

Additional Comments

Please use this space if you would like to note down any additional comments about how your muscle condition affects any of your **day to day, work, or social and leisure activities .**

SECTION B:- RELATIONSHIPS AND SOCIAL INTERACTION.

This next group of questions has been designed to find out about the impact of your muscle condition upon your relationships with other people. You will be asked about:

a) **Your relationships with friends**
b) **Your relationships with family members**
c) **Your relationship with your partner/spouse**
d) **Your contact with other people in general (e.g. with strangers, acquaintances and colleagues).**

Question 4

a) Are your relationships with your friends affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF THEY ARE NOT AFFECTED, PLEASE GO TO QUESTION 5

b) If your relationships with your friends **are** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your relationships with your friends?

not at all important	quite important	moderately important	very important	extremely important

Question 5

a) Are your family relationships affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF THEY ARE NOT AFFECTED, PLEASE GO TO QUESTION 6

b) If your family relationships **are** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your family relationships?

not at all important	quite important	moderately important	very important	extremely important

Question 6

a) Is your relationship with your partner/spouse affected by your muscle condition?

IF THIS QUESTION DOES NOT APPLY TO YOU TICK THIS BOX ☐ AND GO TO QUESTION 7.

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO QUESTION 7

b) If your relationship with your partner/spouse **is** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your relationship with your partner/spouse?

not at all important	quite important	moderately important	very important	extremely important

Question 7

a) Is your day to day contact with other people (e.g. how you get along with strangers, acquaintances, colleagues) affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO THE COMMENTS SECTION, JUST BEFORE SECTION C.

b) If your day to day contact with other people **is** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your day to day contact with other people?

not at all important	quite important	moderately important	very important	extremely important

Additional Comments

Please use this space if you would like to note down any additional comments about how your muscle condition affects any of your **relationships, or your contact with other people in general.**

SECTION C:- PERSONAL FEELINGS.

This next group of questions, starting on the following page has been designed to find out about the impact of your muscle condition upon your personal feelings about yourself and about the future. You will be asked about:

a) **Your independence**
b) **Your emotions**
c) **Your feelings about your physical appearance**
d) **Your feelings about the future**

Question 8

a) Is your independence affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO QUESTION 9

b) If your independence **is** affected, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your independence?

not at all important	quite important	moderately important	very important	extremely important

Question 9

a) Does your muscle condition affect how you feel emotionally?

not at all	slightly	moderately	quite a lot	very much

IF YOU ARE NOT AFFECTED, PLEASE GO TO QUESTION 10

b) If you **are** affected emotionally, overall is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon how you feel emotionally?

not at all important	quite important	moderately important	very important	extremely important

Question 10

a) Does your muscle condition affect how you feel about the way you look?

not at all	slightly	moderately	quite a lot	very much

IF YOU ARE NOT AFFECTED, PLEASE GO TO QUESTION 11

b) If how you feel about the way you look **is** affected, is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon how you feel about the way you look?

not at all important	quite important	moderately important	very important	extremely important

Question 11

a) Is your idea of the future affected by your muscle condition?

not at all	slightly	moderately	quite a lot	very much

IF IT IS NOT AFFECTED, PLEASE GO TO THE COMMENTS SECTION, JUST BEFORE SECTION D.

b) If your idea of the future **is** affected, is this effect:

Good ☐ Bad ☐ Other (Please specify_____) ☐

c) How important to you is the effect of your muscle condition upon your idea of the future?

not at all important	quite important	moderately important	very important	extremely important

Additional Comments.

Please use this space if you would like to note down any additional comments about how your muscle condition affects **how you feel about yourself in terms of your independence, your emotions, the way you look, or your idea of the future.**

SECTION D:- TREATMENT.

This question is designed to find out about how the treatment you receive for your muscle condition affects your life.

Question 12A

Do you receive treatment (for example,tablets and/or injections) for your muscle condition?

Yes ☐ IF "YES", GO TO QUESTION 12B

No ☐ IF "NO", GO TO QUESTION 12C

Question 12B

a) Does the treatment you receive for your muscle condition have any **good** effects?

none at all	some	moderate	quite a lot	many

IF IT DOES NOT HAVE ANY GOOD EFFECTS, PLEASE GO TO PART (C) OF THIS QUESTION

b) How important to you are the **good** effects of the treatment for your muscle condition?

not at all important	quite important	moderately important	very important	extremely important

a) Does the treatment you receive for your muscle condition have any **bad** effects?

none at all	some	moderate	quite a lot	many

IF IT DOES NOT HAVE ANY BAD EFFECTS, PLEASE GO TO THE "ADDITIONAL COMMENTS" SECTION (TOP OF NEXT PAGE)

b) How important to you are the **bad** effects of the treatment for your muscle condition?

not at all important	quite important	moderately important	very important	extremely important

Additional Comments.

Please use this space if you would like to note down any additional comments about how the treatment for your muscle condition affects your life.

Question 12C.

PLEASE ONLY ANSWER THIS QUESTION IF YOU DO NOT RECEIVE TREATMENT FOR YOUR CONDITION.

a) For what reason **don't** you receive treatment for your muscle condition?

There is no treatment for my muscle condition ☐

The treatment I received in the past did not work ☐

Stopped treatment because of side effects ☐

Other (Please specify_____) ☐

b) How does not receiving treatment for your muscle condition make you feel?

PLEASE WRITE IN THE SPACE BELOW

PLEASE TURN OVER

Section E: Additional Comments.

Please use this space if you would like to note down any other ways in which you feel your muscle condition affects your life, or any additional comments you would like to make.

PLEASE MAKE SURE YOU HAVE COMPLETED ALL PARTS OF THE QUESTIONNAIRE THAT APPLY TO YOU AND THEN FILL IN THE DETAILS BELOW.

Today's Date: ___/____/____

Date of birth: ___/____/____

Sex: Male ☐ Female ☐

What sort of muscle condition do you have (if known) :

_____

How long have you had this muscle condition:

_____

Who do you see about your muscle condition?

A specialist ☐

A GP (General practitioner) ☐

Other (Please specify_____) ☐

Thank you very much for your time.

HOW YOUR MUSCLE CONDITION AFFECTS YOU

This questionnaire is designed to see how your muscle condition affects you.
You will be asked about your symptoms, how bad you feel they are, and how you expect treatment, if you receive any, to affect your symptoms.
You will then be asked how each symptom affects your activities, relationships and your emotions.

There will be a section on how you feel about your physical ability, your relationships and your emotions in relation to how you would like them to be.
The last section will ask about any side effects you have had or expect to have due to treatment.

The information you provide will help doctors to understand your problems.
This will mean they can work towards better care and treatment for you.

To answer the questions either tick boxes or to circle a number from 0 to 10.
For the 0-10 scale questions '0' indicates no effect, and '10' indicates the greatest effect.

QUESTION 1:- THE WEAKNESS OF YOUR MUSCLES

This set of questions will ask you about any weakness you have due to your muscle condition and how much it affects the different areas of your life.
By weakness we mean muscle weakness affecting not only your legs and arms but other muscles such as the those affecting your grip, swallowing, eyes and face, bladder and bowel control, etc.

1A a) Have you had any weakness **over the last two weeks** as a result of your muscle condition?

NO	
YES	

→ PLEASE GO TO QUESTION 2 (PAGE 4).

b) How bad has this weakness been **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
No weakness										Extreme weakness

1c) How do you expect treatment to affect your **muscle weakness?**

Tick here if you do **not** receive treatment for your muscle condition at the moment ☐

PLEASE TICK ONE BOX

It will have no effect on my muscle weakness	
It will slow down the progression of my weakness	
It will stop the progression of my weakness	
It will improve my strength	
It will completely bring back my strength	
I have not thought about it	

1B. THE THINGS YOU DO

Has your **muscle weakness** made the following activities difficult, **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Daily activities (for example, washing, dressing & housework)	0	1	2	3	4	5	6	7	8	9	10
Work activities	0	1	2	3	4	5	6	7	8	9	10
Please tick if you are unemployed/retired at the moment ☐											
Leisure activities	0	1	2	3	4	5	6	7	8	9	10

1C. YOUR RELATIONSHIPS WITH OTHER PEOPLE

Has your **muscle weakness** made relationships with the following people difficult **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Partner/ spouse	0	1	2	3	4	5	6	7	8	9	10
Please tick if not applicable ☐											
Other family	0	1	2	3	4	5	6	7	8	9	10
Friends	0	1	2	3	4	5	6	7	8	9	10
Other people (for example colleagues acquaintances & strangers)	0	1	2	3	4	5	6	7	8	9	10

1D. HOW YOU FEEL

Over the **last 2 weeks** has your **muscle weakness** made you feel:

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Anxious/worried	0	1	2	3	4	5	6	7	8	9	10
Depressed	0	1	2	3	4	5	6	7	8	9	10
Frustrated	0	1	2	3	4	5	6	7	8	9	10
Low in confidence/ self-esteem	0	1	2	3	4	5	6	7	8	9	10

QUESTION 2:- THE STIFFNESS/ 'LOCKING' OF YOUR MUSCLES.

This set of questions will ask you about any muscle stiffness/ 'locking' of your muscles you have due to your muscle condition. You will then be asked how much this affects the different areas of your life.

2A a) Have you had any stiffness/ 'locking' of your muscles **over the last two weeks** as a result of your muscle condition?

NO	
YES	

→ PLEASE GO TO QUESTION 3 (PAGE 6).

b) How bad has this stiffness/ 'locking' been **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
No stiffness/'locking'										Extreme stiffness/'locking'

c) How do you think treatment will affect the **stiffness/ 'locking' of your muscles?**

Tick here if you do **not** receive treatment for your muscle condition at the moment ☐

PLEASE TICK ONE BOX

It will have no effect upon the stiffness/ 'locking' of my muscles	
It will slow down any increase in the stiffness/ 'locking' of my muscles	
It will stop the stiffness/ 'locking' from becoming worse	
It will partly relieve the stiffness/ 'locking' of my muscles	
It will completely relieve the stiffness/ 'locking' of my muscles	
I have not thought about it	

2A. THE THINGS YOU DO

Has **the stiffness/ 'locking' of your muscles** made the following activities difficult, **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Daily activities (for example, washing, dressing & housework)	0	1	2	3	4	5	6	7	8	9	10
Work activities Please tick if you are unemployed/retired at the moment ☐	0	1	2	3	4	5	6	7	8	9	10
Leisure activities	0	1	2	3	4	5	6	7	8	9	10

2B. YOUR RELATIONSHIPS WITH OTHER PEOPLE

Has the **stiffness/ 'locking' of your muscles** made relationships with the following people difficult **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Partner/ spouse Please tick if not applicable ☐	0	1	2	3	4	5	6	7	8	9	10
Other family	0	1	2	3	4	5	6	7	8	9	10
Friends	0	1	2	3	4	5	6	7	8	9	10
Other people (for example colleagues acquaintances & strangers)	0	1	2	3	4	5	6	7	8	9	10

2D. HOW YOU FEEL

Over the **last 2 weeks** has the **stiffness/ 'locking' of your muscles** made you feel:

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all much									Very
Anxious/worried	0	1	2	3	4	5	6	7	8	9	10
Depressed	0	1	2	3	4	5	6	7	8	9	10
Frustrated	0	1	2	3	4	5	6	7	8	9	10
Low in confidence/ self-esteem	0	1	2	3	4	5	6	7	8	9	10

QUESTION 3:- YOUR PAIN

This set of questions will ask you about any pain you have due to your muscle condition and how much this affects the different areas of your life.

3A a) Have you had pain **over the last two weeks** as a result of your muscle condition?

NO	
YES	

→ PLEASE GO TO QUESTION 4 (PAGE 8).

b) How bad has this pain been **over the last two weeks**, on a scale of one to ten?

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
No pain										Extreme pain

c) How do you think treatment will affect your **pain**?

Tick here if you do **not** and **will not soon** receive treatment for your muscle condition ☐

PLEASE TICK ONE BOX

It will have no effect on my pain	
It will slow down any increase in my pain	
It will stop my pain from getting worse	
It will partly relieve my pain	
It will completely relieve my pain	
I have not thought about it	

3B. THE THINGS YOU DO

Has your **pain** made the following activities difficult, **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Daily activities (for example, washing, dressing & housework)	0	1	2	3	4	5	6	7	8	9	10
Work activities Please tick if you are unemployed/retired at the moment ☐	0	1	2	3	4	5	6	7	8	9	10
Leisure activities	0	1	2	3	4	5	6	7	8	9	10

3C. YOUR RELATIONSHIPS WITH OTHER PEOPLE

Has your pain made relationships with the following people difficult **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Partner/ spouse Please tick if not applicable ☐	0	1	2	3	4	5	6	7	8	9	10
Other family	0	1	2	3	4	5	6	7	8	9	10
Friends	0	1	2	3	4	5	6	7	8	9	10
Other people (for example colleagues acquaintances & strangers)	0	1	2	3	4	5	6	7	8	9	10

3D. HOW YOU FEEL

Over the last 2 weeks has your pain made you feel:

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all much									Very
Anxious/worried	0	1	2	3	4	5	6	7	8	9	10
Depressed	0	1	2	3	4	5	6	7	8	9	10
Frustrated	0	1	2	3	4	5	6	7	8	9	10
Low in confidence/ self-esteem	0	1	2	3	4	5	6	7	8	9	10

QUESTION 4: - HOW TIRED YOU FEEL

This set of questions will ask you about any tiredness/fatigue you have due to your muscle condition and how much this affects the different areas of your life.

4A a) Have you felt tired/fatigued **over the last two weeks** as a result of your muscle condition?

NO	
YES	

→ PLEASE GO TO QUESTION 5 (PAGE 10).

b) How bad has your tiredness/fatigue been **over the last two weeks,** on a scale of one to ten?

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
No fatigue										Extreme fatigue

c) How do you think treatment will affect your **tiredness/fatigue?**

Tick here if you do **not** and **will not soon** receive treatment for your muscle condition ☐

PLEASE TICK ONE BOX

No effect upon my fatigue	
Slow down any increase in my fatigue	
Stop my fatigue from becoming worse	
Increase the energy I have	
Completely bring back my energy	
Not thought about it	

4B. THE THINGS YOU DO.

Has your **tiredness/fatigue** made the following activities difficult, **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Daily activities (for example, washing, dressing & housework)	0	1	2	3	4	5	6	7	8	9	10
Work activities Please tick if you are unemployed/retired at the moment ☐	0	1	2	3	4	5	6	7	8	9	10
Leisure activities	0	1	2	3	4	5	6	7	8	9	10

4C. YOUR RELATIONSHIPS WITH OTHER PEOPLE

Has your tiredness/fatigue made your relationships with the following people difficult **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Partner/ spouse Please tick if not applicable ☐	0	1	2	3	4	5	6	7	8	9	10
Other family	0	1	2	3	4	5	6	7	8	9	10
Friends	0	1	2	3	4	5	6	7	8	9	10
Other people (for example colleagues acquaintances & strangers)	0	1	2	3	4	5	6	7	8	9	10

4D. HOW YOU FEEL

Over the **last 2 weeks** has your **tiredness/fatigue** made you feel:

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all much									Very
Anxious/worried	0	1	2	3	4	5	6	7	8	9	10
Depressed	0	1	2	3	4	5	6	7	8	9	10
Frustrated	0	1	2	3	4	5	6	7	8	9	10
Low in confidence/ self-esteem	0	1	2	3	4	5	6	7	8	9	10

QUESTION 5: - THE WAY YOU LOOK

This set of questions will ask you whether your muscle condition affects the way your look. and how much this affects the different areas of your life.

5A a) Does your muscle condition affect the way you look?

NO	
YES	

→ PLEASE GO TO QUESTION 6 (PAGE 12).

b) How badly has your muscle condition affected the way you look over the last two weeks?

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
No effect effect										Extreme

c) How do you think treatment will affect **the way you look**?

Tick here if you do **not** and **will not soon** receive treatment for your muscle condition ☐

PLEASE TICK ONE BOX

It will have no effect upon the way I look	
It will slow down the effects of my muscle condition upon the way I look	
It will stop any further effects of my muscle condition upon the way I look	
It will improve the way I look	
It will greatly improve the way I look	
I have not thought about it	

5B. THE WAY YOU LOOK.

Has **the way you look** made the following activities difficult, **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Daily activities (for example, washing, dressing & housework)	0	1	2	3	4	5	6	7	8	9	10
Work activities Please tick if you are unemployed/retired at the moment ☐	0	1	2	3	4	5	6	7	8	9	10
Leisure activities	0	1	2	3	4	5	6	7	8	9	10

5C. YOUR RELATIONSHIPS WITH OTHER PEOPLE

Has **the way you look** made relationships with the following people difficult **over the last two weeks?**

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Partner/ spouse Please tick if not applicable ☐	0	1	2	3	4	5	6	7	8	9	10
Other family	0	1	2	3	4	5	6	7	8	9	10
Friends	0	1	2	3	4	5	6	7	8	9	10
Other people (for example colleagues acquaintances & strangers)	0	1	2	3	4	5	6	7	8	9	10

5D. HOW YOU FEEL

Over the **last 2 weeks** has **the way you look** made you feel:

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all									Very much
Anxious/worried	0	1	2	3	4	5	6	7	8	9	10
Depressed	0	1	2	3	4	5	6	7	8	9	10
Frustrated	0	1	2	3	4	5	6	7	8	9	10
Low in confidence/ self-esteem	0	1	2	3	4	5	6	7	8	9	10

QUESTION 6: YOUR EXPECTATIONS

6A a) My ability to do all the things I want to do is:

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
Exactly as I want it to be										The worst it could possibly be

b) How important is this to you:

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
Not important										Extremely important

6B a) My relationships with other people are:

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
Exactly as I want them to be										The worst it could possibly be

b) How important is this to you?

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
Not important										Extremely important

6C a) The way I feel emotionally is

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
Exactly as I want it to be										The worst it could possibly be

b) How important is this to you?

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
Not important										Extremely important

6D a) My level of independence is

PLEASE CIRCLE ONE NUMBER

0	1	2	3	4	5	6	7	8	9	10
Exactly as I want it to be										The worst it could possibly be

b) How important is this to you?

PLEASE CIRCLE ONE NUMBER

Not important										Extremely important
0	1	2	3	4	5	6	7	8	9	10

Question 7.

7A. Do you receive treatment for your muscle condition

NO	
YES	

→ PLEASE GO TO THE NEXT PAGE (PAGE 14).

7B. Have you had any problems with your treatment?

NO	
YES	

PLEASE LIST ANY PROBLEMS YOU HAVE HAD WITH YOUR TREATMENT

7C. Do you expect to experience any problems with treatment?

NO	
YES	

PLEASE LIST ANY PROBLEMS YOU EXPECT TO HAVE WITH TREATMENT

Comments

If you have any comments you would like to make about your condition and the way it affects you, please use the space below.

THANK YOU VERY MUCH FOR YOUR HELP

Appendix E: Individualised Neuromuscular Quality of Life Questionnaire (INQoL)

HOW YOUR MUSCLE CONDITION AFFECTS YOU

This questionnaire is designed to see how your muscle condition affects you.

You will be asked about your symptoms and how much they affect your life. There will be questions asking how you feel about your physical ability, independence, relationships, how you feel emotionally and the way you look.
The last section will ask about any treatment you might receive. You will be asked about the effects this treatment has had and the effects you expect it to have.

The information you provide will help doctors to understand your problems. This will mean they can work towards better care and treatment for you.

Please read the questions carefully and answer <u>all</u> the questions that apply to you. Thank you.

QUESTION 1:- YOUR MUSCLE WEAKNESS

1 Do you have any muscle weakness due to your muscle condition?
By weakness we mean any weakness in your legs, arms and hands or in any other muscles. For example, your face, eyes, swallowing, breathing or bladder and bowel control may be affected.

PLEASE TICK ONE BOX

NO	
YES	

→ PLEASE GO TO **QUESTION 2A** (NEXT PAGE)

a) How much weakness would you say you have in the muscles affected by your condition?

PLEASE CIRCLE ONE NUMBER

Very little	Some	A fair amount	A moderate amount	A considerable amount	A lot	An extreme amount
1	2	3	4	5	6	7

b) Does your muscle weakness cause difficulties in your life at the moment?

PLEASE CIRCLE ONE NUMBER

None at all	Some	A fair amount	A moderate amount	A considerable amount	Very many	An extreme amount
0	1	2	3	4	5	6

c) How important to you are any difficulties caused by your muscle weakness?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

QUESTION 2:- THE 'LOCKING' OF YOUR MUSCLES

2 Do you have any 'locking' (seizing up) of your muscles as a result of your muscle condition?

PLEASE TICK ONE BOX

NO	
YES	

→ PLEASE GO TO **QUESTION 3A** (NEXT PAGE)

a) How much muscle 'locking' would you say you have at the moment?

PLEASE CIRCLE ONE NUMBER

Very little	Some	A fair amount	A moderate amount	A considerable amount	A lot	An extreme amount
1	2	3	4	5	6	7

b) Does the 'locking' of your muscles cause difficulties in your life at the moment?

PLEASE CIRCLE ONE NUMBER

None at all	Some	A fair amount	A moderate amount	A considerable amount	Very many	An extreme amount
0	1	2	3	4	5	6

c) How important to you are any difficulties caused by the 'locking' of your muscles?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

QUESTION 3:- YOUR PAIN

3 Do you have any pain as a result of your muscle condition?

PLEASE TICK ONE BOX

NO	
YES	

→ PLEASE GO TO **QUESTION 4A** (NEXT PAGE)

a) How much pain would you say you have at the moment?

PLEASE CIRCLE ONE NUMBER

Very little	Some	A fair amount	A moderate amount	A considerable amount	A lot	An extreme amount
1	2	3	4	5	6	7

b) Does your pain cause difficulties in your life at the moment?

PLEASE CIRCLE ONE NUMBER

None at all	Some	A fair amount	A moderate amount	A considerable amount	Very many	An extreme amount
0	1	2	3	4	5	6

c) How important to you are any difficulties caused by your pain ?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

QUESTION 4:- HOW TIRED YOU FEEL

4 Do you feel tired/ fatigued as a result of your muscle condition?

PLEASE TICK ONE BOX

NO	
YES	

→ PLEASE GO TO **QUESTION 5** (NEXT PAGE)

a) How much tiredness/ fatigue would you say you have at the moment?

PLEASE CIRCLE ONE NUMBER

Very little	Some	A fair amount	A moderate amount	A considerable amount	A lot	An extreme amount
1	2	3	4	5	6	7

b) Does your tiredness/ fatigue cause difficulties in your life at the moment?

PLEASE CIRCLE ONE NUMBER

None at all	Some	A fair amount	A moderate amount	A considerable amount	Very many	An extreme amount
0	1	2	3	4	5	6

c) How important to you are any difficulties caused by your tiredness/ fatigue?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

QUESTION 5: THE THINGS YOU DO

5A At the moment, does your muscle condition affect your ability to do the following activities?

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all	Slightly	A fair amount	Moder-ately	Consid-erably	Very much	Extrem-ely
I. Daily activities (for example, washing, dressing & housework)	0	1	2	3	4	5	6
II. Leisure activities	0	1	2	3	4	5	6
III. Work activities	0	1	2	3	4	5	6

If you have **no paid employment** (for example, you are unemployed or retired or you work in the home), please tick here ☐

If you are **not working due to your condition**, please tick here ☐

B I. In the face of my condition, my ability to do all the things I want to do is:

PLEASE CIRCLE ONE NUMBER

Exactly as I would like it to be	Good, but not quite how I would like it to be	OK, but not how I would like it to be	Neither good nor bad	Quite bad, but it could be much worse	Bad, but it could be worse	The worst it could possibly be
0	1	2	3	4	5	6

II. How important to you is the effect of your muscle condition on your ability to do all the things you want to do?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

OR If your ability is '**exactly as you would like**', please tick here ☐

QUESTION 6: YOUR INDEPENDENCE

6A At the moment, how much help do you need from other people in carrying out your activities? (for example, daily activities & going out)

None at all	Some	A fair amount	A moder-ate amount	A consid-erable amount	Very much	An extreme amount
0	1	2	3	4	5	6

B I. In the face of my condition, my level of independence is:

PLEASE CIRCLE ONE NUMBER

Exactly as I would like it to be	Good, but not quite how I would like it to be	OK, but not how I would like it to be	Neither good nor bad	Quite bad, but it could be much worse	Bad, but it could be worse	The worst it could possibly be
0	1	2	3	4	5	6

II. How important to you is the effect of your muscle condition on your level of independence?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

OR If your independence is '**exactly as you would like**', please tick here ☐

QUESTION 7: YOUR RELATIONSHIPS

7A At the moment, does your muscle condition cause any difficulties in your relationships with the following people?

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	None at all	Some	A fair amount	A moder-ate amount	A consid-erable amount	Very many	An extreme amount
I. Partner/ spouse	0	1	2	3	4	5	6
If you are **not married or in a relationship** at the moment or if you are **widowed** please tick here ☐							
II. Other family members	0	1	2	3	4	5	6
III. Friends	0	1	2	3	4	5	6
IV. Other people For example, strangers, acquaintances & colleagues.	0	1	2	3	4	5	6

B I. In the face of my condition, my close family relationships are:

PLEASE CIRCLE ONE NUMBER

Exactly as I would like them to be	Good, but not quite how I would like them to be	OK, but not how I would like them to be	Neither good nor bad	Quite bad, but they could be much worse	Bad, but they could be worse	The worst they could possibly be
0	1	2	3	4	5	6

II. How important to you is the effect of your muscle condition on your close family relationships?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

OR If your close family relationship are **'exactly as you would like'**, please tick here ☐

III. In the face of my condition, my close friendships are:

PLEASE CIRCLE ONE NUMBER

Exactly as I would like them to be	Good, but not quite how I would like them to be	OK, but not how I would like them to be	Neither good nor bad	Quite bad, but they could be much worse	Bad, but they could be worse	The worst they could possibly be
0	1	2	3	4	5	6

IV. How important to you is the effect of your muscle condition on your close friendships?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

OR If your close friendships are '**exactly as you would like**', please tick here ☐

V. In the face of my condition, my relationships with other people (for example, acquaintances, strangers and colleagues) are:

PLEASE CIRCLE ONE NUMBER

Exactly as I would like them to be	Good, but not quite how I would like them to be	OK, but not how I would like them to be	Neither good nor bad	Quite bad, but they could be much worse	Bad, but they could be worse	The worst they could possibly be
0	1	2	3	4	5	6

VI. How important to you is the effect of your muscle condition on your relationships with these other people?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

OR If your relationships with others are '**exactly as you would like**', please tick here ☐

QUESTION 8: HOW YOU FEEL

8A At the moment, does your muscle condition make you feel:

PLEASE CIRCLE ONE NUMBER FOR EACH ITEM

	Not at all	Slightly	A fair bit	Moder-ately	Consid-erably	Very much	Extrem-ely
I. Anxious/worried	0	1	2	3	4	5	6
II. Depressed	0	1	2	3	4	5	6
III. Frustrated	0	1	2	3	4	5	6
IV. Low in confidence/ self-esteem	0	1	2	3	4	5	6

B I. In the face of my condition, the way I feel emotionally is:

PLEASE CIRCLE ONE NUMBER

Exactly as I would like to be	Good, but not quite how I would like to be	OK, but not how I would like to be	Neither good nor bad	Quite bad, but I could be much worse	Bad, but I could be worse	The worst I could possibly be
0	1	2	3	4	5	6

II, How important to you is the effect of your muscle condition upon the way you feel emotionally?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

OR If the way you feel emotionally is **'exactly as you would like'**, please tick here ☐

QUESTION 9:- THE WAY YOU LOOK

9A At the moment, does your muscle condition affect the way you look?
Your muscle condition might affect the way your body, face or skin looks or perhaps the way you move or whether to need to use a stick or wheelchair.

PLEASE CIRCLE ONE NUMBER

Not at all	Slightly	A fair amount	A moder-ate amount	A consid-erable amount	Very much	An extreme amount
0	1	2	3	4	5	6

B I. In the face of my condition, the way I look is:

PLEASE CIRCLE ONE NUMBER

Exactly as I would like to be	Good, but not quite how I would like to be	OK, but not how I would like to be	Neither good nor bad	Quite bad, but it could be much worse	Bad, but it could be worse	The worst it could possibly be
0	1	2	3	4	5	6

II. How important to you is the effect of your muscle condition upon the way you look?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

OR If the way you look is **'exactly as you would like'**, please tick here ☐

QUESTION 10: TREATMENT

10A Do you receive, or are you about to start receiving treatment for your muscle condition? (For example, tablets, injections or physiotherapy)

PLEASE TICK ONE BOX

NO	→ PLEASE GO TO **PAGE 14** (LAST PAGE)
YES	→

I. Do you feel the treatment you receive for your muscle condition has had beneficial effects?

PLEASE CIRCLE ONE NUMBER

None at all	Some	A fair amount	A moder-ate amount	A consid-erable amount	Very many	An extreme amount
0	1	2	3	4	5	6

If you are **not yet receiving treatment,** please tick here ☐
If you are **unsure**, please tick here ☐

II. Do you feel the treatment you receive for your muscle condition will have beneficial effects in the future?

PLEASE CIRCLE ONE NUMBER

None at all	Some	A fair amount	A moder-ate amount	A consid-erable amount	Very many	An extreme amount
0	1	2	3	4	5	6

If you are **unsure**, or if you have **not thought about it** please tick here ☐

III. How important to you are the beneficial effects of treatment?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

10B I. Do you feel the treatment you receive for your muscle condition has had harmful side effects?

PLEASE CIRCLE ONE NUMBER

None at all	Some	A fair amount	A moder-ate amount	A consid-erable amount	Very many	An extreme amount
0	1	2	3	4	5	6

If you are **not yet receiving treatment,** please tick here ☐

If you are **unsure,** please tick here ☐

II. Do you think the treatment you receive for your muscle condition will have side effects in the future?

PLEASE CIRCLE ONE NUMBER

None at all	Some	A fair amount	A moder-ate amount	A consid-erable amount	Very many	An extreme amount
0	1	2	3	4	5	6

If you are **unsure,** or if you have **not thought about it** please tick here ☐

III. How important to you are the side effects of treatment?

PLEASE CIRCLE ONE NUMBER

Not at all important	Quite important	Reason-ably important	Moderate-ly important	Consider-ably important	Very important	Extremely important
0	1	2	3	4	5	6

PLEASE TURN OVER

Comments

If you have any comments you would like to make about your condition and the way it affects you, please use the space below.

Your name (please print): _____

Today's date: ___/___/____

THANK YOU VERY MUCH FOR YOUR HELP

Appendix F: Missing data. Instructions for scoring

Quns 1-4

1A-4A If missing and rest of symptom qun missing (i.e. part B) -> score zero

1B-4 B a If missing, score '1'
b If missing, impute previous value (part a)
c If missing, score '0'

Qun 5

5 A If an item item missing, sum completed items, and multiply by 6 if two items completed and 12 if only one item has been completed (to get score out of 72)
(N.B. 'Work activities' item: if not working due to condition score '6' and count as a completed item).

EXAMPLE: If 'Leisure activities' is missing, add values of 'Daily activities' & 'Work Activities'. Multiply this by 6.

BI If missing, impute average of completed 'activities' items (from 5A)
BII If missing, score as '0'

Qun 6

6 A If missing, score as '0'
BI If missing, impute value from 6A
BII If missing, score as '0'

Qun 7

7 A If item missing, sum the completed items and multiply by 4 if three completed items, 6 if two completed items and 12 if one completed item.

BI If missing, impute average of 7a items –
i) Relationship with spouse/partner &
ii) Relationship with other family members.
BII If missing, score as '0'
BIII If missing, impute value from 'Friends' (7AIII) item
BIV If missing, score as '0'
BV If missing, impute value from 'Other people' (7AIV) item
BVI If missing, score as '0'

Qun 8

8 A If item missing, sum the completed items and multiply by 4 if three completed items, 6 if two completed items and 12 if one completed item.

BI If missing, impute average of items completed in 8A

BII If missing, score as '0'

Qun 9

9 A If missing, score as '0'
BI If missing, impute value from 9a
BII If missing, score as '0'

Qun 10

10A I If missing, score as '0'
II If missing, score as '0'
III If missing, score as '0'

10B I If missing, score as '0'
II If missing, score as '0'
III If missing, score as '0'

Appendix G: INQoL Scoring Scheme and Profile Sheet

1. **Weakness score = (a+b+c) / 19 X 100**
2. **Muscle 'locking' score = (a+b+c) / 19 X 100**
3. **Pain score = (a+b+c) / 19 X 100**
4. **Fatigue score = (a+b+c) / 19 X 100**
5. **Activities score = [4 X (AI+AII+AIII)] + [3 X (BI+BII)] / 108 X 100**

If not working due to condition item → AIII=6
If retired/unemployed/work in home (not as a result of condition)
→ [6 X (AI+AII)] + [3 X (BI+BII)] /108 x 100

6. **Independence score = [12 X A] + [3 X (BI+BII)] /108 x 100**
7. **Social relationships = [3X(AI+AII+AIII+AIV)+BI+II+III+IV+V+VI] / 108 x 100**

If partner/spouse item(AI) not applicable :
→ [4 X (AII+AIII+AIV)] + [BI+II+III+IV+V+VI] / 108 x 100

8. **Emotions score = [3 X (AI+AII+AIII)] + [3 X (BI+BII)] / 108 x 100**
9. **Body Image score = [12 X (A)] + [3 X (BI+BII)] / 108 X 100**
10. **QoL score**

Add scores of items in section B for questions 5-9, divide total score by 180 and multiply by 100 (to achieve percentage score)

11. **Perceived Treatment effects = [(AI+AIII) – (BI+BIII)] / 12 x 100**
    Expected treatment effects = [(AII+AIII) – (BII+BIII)] / 12 x 100

INQoL Profile

Symptoms	Scores (0-100)
Weakness	
'Locking'	
Pain	
Fatigue	

Life Domains	Scores (0-100)
Activities	
Independence	
Social Relationships	
Emotions	
Body Image	

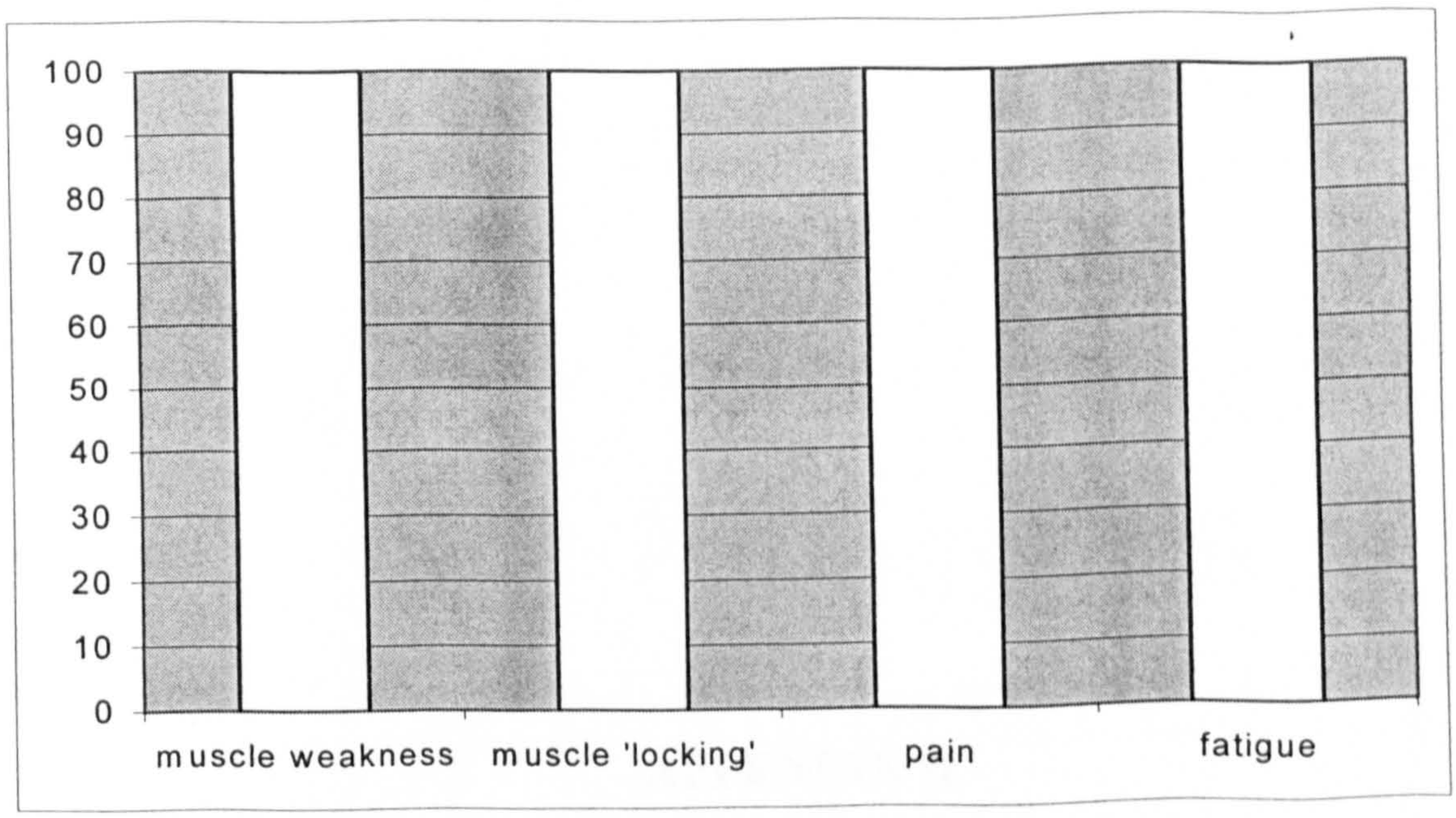
QoL	Score (0-100)
NMD-related QoL	

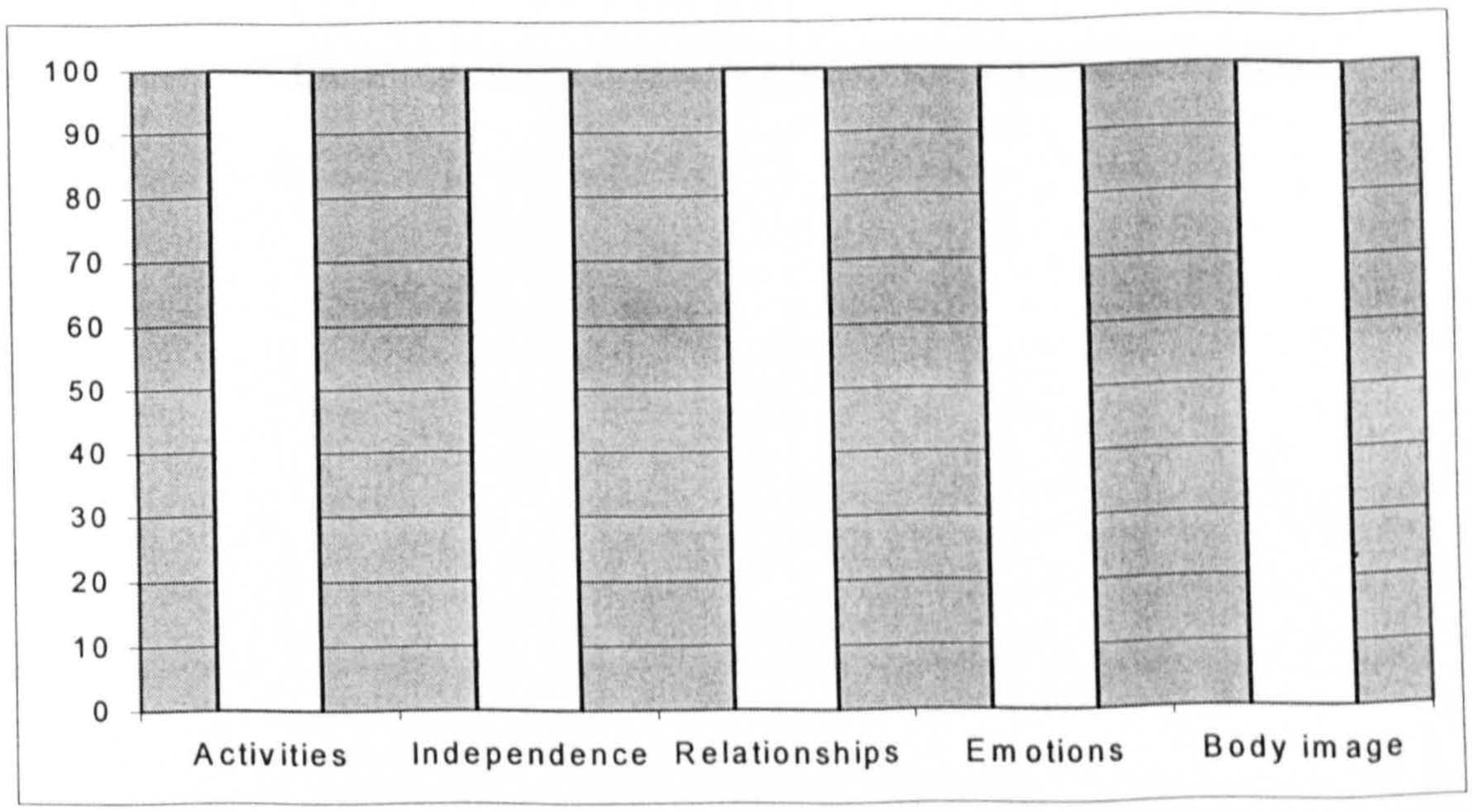
Treatment effects	Scores (-100 to +100)
Perceived treatment effects	
Expected treatment effects	

Individualised Neuromuscular Quality of Life Profile Date _____

Patient Name _____

Higher scores indicate greater impact





Perceived treatment effects (-100 to +100)	
Expected treatment effects (-100 to +100)	

Negative impact upon quality of life (100 maximum score possible)	

Comments:

APPENDIX H

Questionnaires Incorporated in the Validation Process

The Nottingham Health Profile

Listed below are some problems people might have in their daily lives.

Read the list carefully and put a tick in the box under **Yes** for any problem that applies to you **at the moment**. Tick the box under **No** for any problem that does not apply to you.

Please answer every question. If you are not sure whether to answer yes or no, tick whichever answer you think is **most** true at the moment.

	Yes	No
I'm tired all the time	☐	☐
I have pain at night	☐	☐
Things are getting me down	☐	☐

	Yes	No
I have unbearable pain	☐	☐
I take tablets to help me sleep	☐	☐
I've forgotten what it's like to enjoy myself	☐	☐

	Yes	No
I'm feeling on edge	☐	☐
I find it painful to change position	☐	☐
I feel lonely	☐	☐

Please turn over

	Yes	No
I can only walk about indoors	☐	☐
I find it hard to bend	☐	☐
Everything is an effort	☐	☐

	Yes	No
I'm waking up in the early hours of the morning	☐	☐
I'm unable to walk at all	☐	☐
I'm finding it hard to make contact with people	☐	☐

Remember, if you are not sure whether to answer yes or no to a problem, tick whichever answer you think **more true** at the moment.

	Yes	No
The days seem to drag	☐	☐
I have trouble getting up and down stairs or steps	☐	☐
I find it hard to reach for things	☐	☐

	Yes	No
I'm in pain when I walk	☐	☐
I lose my temper easily these days	☐	☐
I feel there is nobody I am close to	☐	☐

Please turn over

	Yes	No
I lie awake for most of the night	☐	☐
I feel as if I'm losing control	☐	☐
I'm in pain when I'm standing	☐	☐

	Yes	No
I find it hard to dress myself	☐	☐
I soon run out of energy	☐	☐
I find it hard to stand for long (e.g. at the kitchen sink, waiting for a bus)	☐	☐

	Yes	No
I'm in constant pain	☐	☐
It takes me a long time to get to sleep	☐	☐
I feel I am a burden to people	☐	☐

	Yes	No
Worry is keeping me awake at night	☐	☐
I feel that life is not worth living	☐	☐
I sleep badly at night	☐	☐

Please turn over

	Yes	No
I'm finding it hard to get on with people	☐	☐
I need help to walk about outside (e.g. a walking aid or someone to support me)	☐	☐

	Yes	No
I'm in pain when going up and down stairs or steps	☐	☐
I wake up feeling depressed	☐	☐
I'm in pain when I'm sitting	☐	☐

Now please go back to page 1 and make sure that you have answered "**Yes**" or "**No**" to every question, on all four pages of the questionnaire.

Thank you for your help

SF-36 HEALTH SURVEY

INSTRUCTIONS: This survey asks for your views about your health. This information will help keep track of how you feel and how well you are able to do your usual activities.

Answer every question by marking the answer as indicated. If you are unsure about how to answer a question, please give the best answer you can.

1. In general, would you say your health is:

(circle one)

Excellent	1
Very good	2
Good	3
Fair	4
Poor	5

2. Compared to one year ago, how would you rate your health in general now?

(circle one)

Much better now than one year ago	1
Somewhat better now than one year ago	2
About the same as one year ago	3
Somewhat worse now than one year ago	4
Much worse now than one year ago	5

3. The following questions are about activities you might do during a typical day. Does your health now limit you in these activities? If so, how much?

(circle one number on each line)

ACTIVITIES	Yes, Limited A Lot	Yes, Limited A Little	No, Not Limited At All
a. **Vigorous activities**, such as running, lifting heavy objects, participating in strenuous sports	1	2	3
b. **Moderate activities**, such as moving a table, pushing a vacuum cleaner, bowling, or playing golf	1	2	3
c. Lifting or carrying groceries	1	2	3
d. Climbing **several** flights of stairs	1	2	3
e. Climbing **one** flight of stairs	1	2	3
f. Bending, kneeling, or stooping	1	2	3
g. Walking **more than a mile**	1	2	3
h. Walking **half a mile**	1	2	3
i. Walking **one hundred yards**	1	2	3
j. Bathing or dressing yourself	1	2	3

4. During the past 4 weeks, have you had any of the following problems with your work or other regular daily activities as a result of your physical health?

(circle one number on each line)

	YES	NO
a. Cut down on the **amount of time** you spent on work or other activities	1	2
b. **Accomplished less** than you would like	1	2
c. Were limited in the **kind** of work or other activities	1	2
d. Had **difficulty** performing the work or other activities (for example, it took extra effort)	1	2

5. During the <u>past 4 weeks</u>, have you had any of the following problems with your work or other regular daily activities <u>as a result of any emotional problems</u> (such as feeling depressed or anxious)?

(circle one number on each line)

	YES	NO
a. Cut down on the **amount of time** you spent on work or other activities	1	2
b. **Accomplished less** than you would like	1	2
c. Didn't do work or other activities as **carefully** as usual	1	2

6. During the <u>past 4 weeks</u>, to what extent has your physical health or emotional problems interfered with your normal social activities with family, friends, neighbours, or groups?

(circle one)

Not at all 1

Slightly 2

Moderately 3

Quite a bit 4

Extremely 5

7. How much <u>bodily</u> pain have you had during the <u>past 4 weeks</u>?

(circle one)

None 1

Very mild 2

Mild 3

Moderate 4

Severe 5

Very severe 6

8. During the <u>past 4 weeks</u>, how much did <u>pain</u> interfere with your normal work (including both work outside the home and housework)?

(circle one)

Not at all 1

A little bit 2

Moderately 3

Quite a bit 4

Extremely 5

9. These questions are about how you feel and how things have been with you <u>during the past 4 weeks</u>. For each question, please give the one answer that comes closest to the way you have been feeling. How much of the time during the <u>past 4 weeks</u> -

(circle one number on each line)

	All of the Time	Most of the Time	A Good Bit of the Time	Some of the Time	A Little of the Time	None of the Time
a. Did you feel full of life?	1	2	3	4	5	6
b. Have you been a very nervous person?	1	2	3	4	5	6
c. Have you felt so down in the dumps that nothing could cheer you up?	1	2	3	4	5	6
d. Have you felt calm and peaceful?	1	2	3	4	5	6
e. Did you have a lot of energy?	1	2	3	4	5	6
f. Have you felt downhearted and low?	1	2	3	4	5	6
g. Did you feel worn out?	1	2	3	4	5	6
h. Have you been a happy person?	1	2	3	4	5	6
i. Did you feel tired?	1	2	3	4	5	6

10. During the <u>past 4 weeks</u>, how much of the time has your <u>physical health or emotional problems</u> interfered with your social activities (like visiting with friends, relatives, etc.)?

(circle one)

All of the time 1

Most of the time 2

Some of the time 3

A little of the time 4

None of the time 5

11. How TRUE or FALSE is <u>each</u> of the following statements for you?

(circle one number on each line)

	Definitely True	Mostly True	Don't Know	Mostly False	Definitely False
a. I seem to get ill more easily than other people	1	2	3	4	5
b. I am as healthy as anybody I know	1	2	3	4	5
c. I expect my health to get worse	1	2	3	4	5
d. My health is excellent	1	2	3	4	5

FUNCTIONAL LIMITATIONS PROFILE (FLP)

We are interested in the activities that you do in carrying on your life and any changes that describe you today that are related to your health.

This questionnaire lists statements that describe things people often do when they are not well. Even if you think you are well, some of these statements may stand out, because they describe you and are related to your health. As you read each statement in the questionnaire, think of yourself today. When you read a statement that **describes you and is related to your health,** place a tick in the box to the right of the statement.

For example:

I am not driving my car	☐

If you have not been driving for some time because of your health and are still not driving today, you should tick this statement. On the other hand, if you never drive or are not driving today because you car is being repaired, you should not tick it. Tick a statement **only** if you are sure it describes you and is due to health.

AMBULATION ITEMS

The following statements describe walking and use of stairs. Remember, think of yourself today.

Only tick an item if it describes you and is due to your health.

1. I walk shorter distances or often stop for a rest.	☐
2. I do not walk up or down hills.	☐
3. I only use stairs with a physical aid; for example, a handrail, stick or crutches.	☐
4. I only go up and down stairs with assistance from someone else.	☐
5. I get about in a wheelchair.	☐
6. I do not walk at all.	☐
7. I walk by myself but with some difficulty; for example, I limp, wobble, stumble or I have a stiff leg.	☐
8. I only walk with help from someone else.	☐
9. I go up and down stairs more slowly; for example, one step at a time or I often have to stop.	☐
10. I do not use stairs at all.	☐
11. I get about only by using a walking frame, crutches, stick, walls, or hold on to furniture.	☐
12. I walk more slowly.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS ON THIS PAGE ☐

BODY CARE AND MOVEMENT ITEMS

The following statements describe how you move about, bath, go to the toilet, dress yourself today.

Only tick an item if it describes you and is due to your health.

13. I make difficult movements with help; for example getting in or out of the bath or car.	☐
14. I do not get in and out of bed or chairs without the help of a person or mechanic aid.	☐
15. I only stand for short periods of time.	☐
16. I do not keep my balance.	☐
17. I move my hands or fingers with some difficulty or limitation.	☐
18. I only stand up with someone's help.	☐
19. I kneel, stoop or bend down only by holding onto something.	☐
20. I am in a restricted position all the time.	☐
21. I am very clumsy.	☐
22. I get in and out of bed or chairs by grasping something for support or by using a stick or walking frame.	☐
23. I stay lying down most of the time.	☐
24. I change position frequently.	☐
25. I hold onto something to move myself around in bed.	☐
26. I do not bathe myself completely; for example, I need help with bathing.	☐
27. I do not bathe myself at all, but am bathed by someone else.	☐
28. I use a bedpan with help.	☐
29. I have trouble putting on my shoes, socks, or stockings.	☐
30. I do not have control of my bladder.	☐
31. I do not fasten my clothing; for example, I require assistance with buttons, zips or shoelaces.	☐
32. I spend most of the time partly dressed or in pyjamas.	☐
33. I do not have control of my bowels.	☐
34. I dress myself, but do so very slowly.	☐
35. I only get dressed with someone's help.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS ON THIS PAGE ☐

MOBILITY ITEMS

These next statements describe how you get about the house and outside.

Only tick an item if it describes you and is due to your health.

36. I only get about in one building.	☐
37. I stay in one room.	☐
38. I stay in bed more.	☐
39. I stay in bed most of the time.	☐
40. I do not use public transport now.	☐
41. I stay at home most of the time.	☐
42. I only go out if there is a lavatory nearby.	☐
43. I do not go into town.	☐
44. I only stay away from home for short periods.	☐
45. I do not get about in the dark or in places that are not lit unless I have someone to help.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS ON THIS PAGE ☐

HOUSEHOLD MANAGEMENT ITEMS

The following statements describe your daily work, around the home. When you answer, think of yourself today.

Only tick an item if it describes you and is due to your health .

46. I only do housework or work around the house for short periods of time or I rest often.	☐
47. I do less of the daily household chores than I would usually do.	☐
48. I do not do any of the daily household chores that I would usually do.	☐
49. I do not do any of the maintenance or repair work that I would usually do in my home or garden.	☐
50. I do not do any of the shopping that I would usually do.	☐
51. I do not do any of the cleaning that I would usually do.	☐
52. I have difficulty using my hands; for example, turning taps, using kitchen gadgets, sewing or doing repairs.	☐
53. I do not do any of the clothes washing that I would usually do.	☐
54. I do not do heavy work around the house.	☐
55. I have given up taking care of personal or household business affairs; for example, paying bills, banking or doing household accounts.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS IN THIS SECTION ☐

RECREATION AND PASTIME ITEMS

The following statements describe the activities you usually do in your spare time, for relaxation, entertainment or just to pass the time. Again, think of yourself today.

Only tick an item if it describes you and is due to your health.

56. I spend shorter periods of time on my hobbies and recreation.	☐
57. I go out less often to enjoy myself.	☐
58. I am cutting down on some of my usual inactive pastimes; for example, I watch TV less, play cards less, or read less.	☐
59. I am not doing any of my usual inactive pastimes; for example, I do not watch TV, play cards, or read.	☐
60. I am doing more inactive pastimes instead of my other usual activities.	☐
61. I take part in fewer community activities.	☐
62. I am cutting down on some of my usual physical recreation or more active pastimes.	☐
63. I am not doing any of my usual physical recreation or more active pastimes.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS ON THIS PAGE ☐

SOCIAL INTERACTION ITEMS

These statements describe your contact with family and friends today.

Only tick an item if it describes you and is due to your health.

64. I go out less often to visit people.	☐
65. I do not go out at all to visit people.	☐
66. I show less interest in other people's problems; for example, I don't listen when they tell me about their problems; I don't offer to help.	☐
67. I am often irritable with those around me; for example, I snap at people or criticise easily.	☐
68. I show less affection.	☐
69. I take part in fewer social activities than I used to; for example, I go to fewer parties or social events.	☐
70. I am cutting down the length of visits with friends.	☐
71. I avoid having visitors.	☐
72. My sexual activity is decreased.	☐
73. I often express concern over what might be happening to my health.	☐
74. I talk less with other people.	☐
75. I make many demands on other people; for example, I insist that they do things for me or tell them how to do things.	☐
76. I stay alone much of the time.	☐
77. I am disagreeable with my family; for example, I act spitefully or stubbornly.	☐
78. I frequently get angry with my family; for example, I hit them, scream or throw things at them.	☐
79. I isolate myself as much as I can from the rest of my family.	☐
80. I pay less attention to the children.	☐
81. I refuse contact with my family; for example, I turn away from them.	☐
82. I do not look after my children or family as well as I usually do.	☐
83. I do not joke with members of my family as much as I usually do.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS ON THIS PAGE ☐

EMOTION ITEMS

The next statements describe your feelings and behaviour. Again, think of yourself today.

Only tick an item if it describes you and is due to your health.

84. I say how bad or useless I am; for example, that I am a burden on others.	☐
85. I laugh or cry suddenly.	☐
86. I often moan and groan because of pain or discomfort.	☐
87. I have attempted suicide.	☐
88. I behave nervously or restlessly.	☐
89. I keep rubbing or holding areas of my body that hurt or are uncomfortable.	☐
90. I am irritable and impatient with myself; for example, I run myself down, I swear at myself, I blame myself for things that happen.	☐
91. I talk hopelessly about the future.	☐
92. I get sudden frights.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS IN THIS SECTION ☐

ALERTNESS ITEMS

93. I am confused and start to do more than one thing at a time.	☐
94. I have more minor accidents; for example, I drop things, I trip and fall, or I bump into things.	☐
95. I react slowly to things that are said or done.	☐
96. I do not finish things I start.	☐
97. I have difficulty reasoning and solving problems; for example, making plans, making decisions, or learning new things.	☐
98. I sometimes get confused; for example, I do not know where I am, who is around, or what day it is.	☐
99. I forget a lot; for example, things that happened recently, where I put things, or to keep appointments.	☐
100. I do not keep my attention on any activity for long.	☐
101. I make more mistakes than usual.	☐
102. I have difficulty doing things which involve thought and concentration.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS ON THIS PAGE ☐

SLEEP AND REST ITEMS

These statements describe your sleep and rest activities today.

Only tick an item if it describes you and is due to your health.

103. I spend much of the day lying down to rest.	☐
104. I sit for much of the day.	☐
105. I sleep or doze most of the time, day and night.	☐
106. I lie down to rest more often during the day.	☐
107. I sit around half asleep.	☐
108. I sleep less at night; for example, I wake up easily, I don't fall asleep for a long time, or I keep waking up.	☐
109. I sleep or doze more during the day.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS IN THIS SECTION ☐

EATING ITEMS

The following statements describe your eating and drinking habits.

Only tick an item if it describes you and is due to your health.

110. I eat much less than usual.	☐
111. I feed myself but only with specially prepared food or special utensils.	☐
112. I eat special or different food; for example, I follow a soft food, bland, low salt, low fat, or low sugar diet.	☐
113. I eat no food at all, but I take liquids.	☐
114. I just pick or nibble at my food.	☐
115. I drink less fluids	☐
116. I feed myself with help from someone else.	☐
117. I do not feed myself at all but have to be fed.	☐
118. I eat no food at all except by tubes or intravenous infusion.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS ON THIS PAGE ☐

COMMUNICATION ITEMS

These statements are about how much you talk to other people and write.
Please think about yourself today.

Only tick an item if it describes you and is due to your health.

119. I have trouble writing or typing.	☐
120. I communicate mostly by nodding my head, pointing, or using sign language, or other gestures.	☐
121. My speech is understood only by a few people who know me well.	☐
122. I often lose control of my voice when I talk; for example, my voice gets louder or softer or changes unexpectedly.	☐
123. I don't write except to sign my name.	☐
124. I carry on a conversation only when very close to other people or looking directly at them.	☐
125. I speak with difficulty; for example, I get stuck for words, I stutter, I stammer, I slur my words.	☐
126. I am understood with difficulty.	☐
127. I do not speak clearly when I am under stress.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS ON THIS PAGE ☐

WORK ITEMS

The next group of statements has to do with any work you usually do other than managing your home. By this we mean anything that you regard as work that you do on a regular basis.

Do you usually do work other than managing your home? YES ☐ NO ☐
IF YES, COMPLETE THE WORK SECTION .

IF NO:
Are you retired? YES ☐ NO ☐

If you are retired, was your retirement due to your health? YES ☐ NO ☐

If you are not retired, but are not working, is this due to your health? YES ☐ NO ☐

IF YES TO EITHER OF THE PREVIOUS 2 STATEMENTS, PLEASE TICK ITEM 128 AND SKIP THE REST OF THE ITEMS IN THIS SECTION.

IF NO, PLEASE SKIP THIS SECTION.

Only tick an item if it describes you and is due to your health.

128. I do not work at all (INCLUDES RETIRED BECAUSE OF HEALTH)	☐
129. I do part of my job at home.	☐
130. I am not getting as much work done as usual.	☐
131. I often get irritable with my workmates; for example, I snap at them or criticise them easily.	☐
132. I work shorter hours.	☐
133. I only do light work.	☐
134. I only work for short periods of time or often stop to rest.	☐
135. I work at my usual job but with some changes; for example, I use different tools or special aids or I swap jobs with someone else.	☐
136. I do not do my job as carefully and accurately as usual.	☐

TICK HERE WHEN YOU HAVE READ ALL STATEMENTS ON THIS PAGE ☐

A Patient Generated Index of Quality of Life.

Your answers to the following steps will tell us how your life is affected by your neuromuscular disease. It will also tell us how you would like to see your life improved.

Step 1: Identifying Areas	Step 2: Scoring each area	Step 3: Spending points
We would like you to think of the most important areas of your life that are affected by your neuromuscular disease. Please write up to FIVE areas in the boxes below.	In this part we would like you to score the areas you mentioned in step 1. This score should show how badly affected you were over the past MONTH. Please score each area out of 10 using this scale: 10= Exactly as you would like to be 9= Close to how you would like to be 8= Very good, but not how you would like to be 7= Good, but not how you would like to be 6= Between good and fair 5= Fair 4= Between poor and fair 3= Poor but not the worst you could imagine 2= Very poor but not the worst you could imagine 1= Close to the worst you could imagine 0= The worst you could imagine	We want you to imagine that any or all of the areas of your life could be improved. You have 14 imaginary points to spend to show which areas you would most like to see improve. Spend more points on areas you would most like to improve and less on areas that are not so important. You don't have to spend points in every area. You can't spend more than 14 points in total.

Please use the last two boxes to score all other areas affected by health problems and all other non-health areas of life

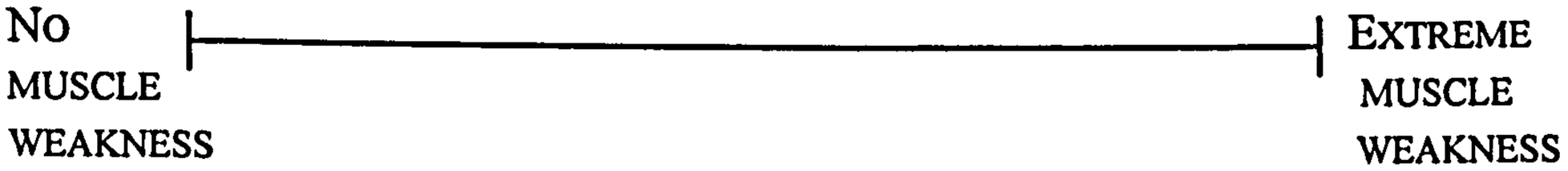
Areas affected by all other health problems

All other non-health areas of life

Remember total must add up to 14

Muscle Weakness Visual Analogue Scale

How bad is your muscle weakness? Please mark an X on the line below, at the point that best describes your level of muscle weakness at the moment.



Muscle 'Locking' Visual Analogue Scale

How bad is your muscle 'locking' (how much do your muscles seize up)? Please mark an X on the line below, at the point that best describes your muscle 'locking' at the moment.

Pain Visual Analogue Scale

How bad is your pain? Please mark an X on the line below, at the point that best describes your level of pain at the moment.

FATIGUE QUESTIONNAIRE (Chalder et al, 1993)

We would like to know whether or not you have been having any problems with feeling tired, weak or lacking in energy in the last few weeks. Please answer ALL the questions simply by underlining or circling the answer which you think most nearly applies to you. We would like to know how you feel either at the moment or recently, rather than a long time ago. If you have been feeling tired for a long time, we want you to compare yourself to how you felt when last well.

Do you have problems with tiredness?	Less than usual	No more than usual	More than usual	Much more than usual
Do you need to rest more?	Less than usual	No more than usual	More than usual	Much more than usual
Do you feel sleepy or drowsy?	Less than usual	No more than usual	More than usual	Much more than usual
Do you have problems starting things?	Less than usual	No more than usual	More than usual	Much more than usual
Do you lack energy?	Better than usual	No more than usual	More than usual	Much more than usual
Do you have less strength in your muscles?	Better than usual	No more than usual	More than usual	Much more than usual
Do you feel weak?	Less than usual	Same as usual	More than usual	Much more than usual
Do you have difficulty concentrating?	Less than usual	Same as usual	More than usual	Much more than usual
Do you make slips of the tongue when speaking?	Less than usual	No more than usual	More than usual	Much more than usual

Do you find it more difficult to find the correct word?	Less than usual	No more than usual	More than usual	Much more than usual
How is your memory?	Better than usual	No worse than usual	Worse than usual	Much worse than usual

The next questions ask about muscle pain.

Do your muscles hurt at rest?	Less than usual	No more than usual	Worse than usual	Much worse than usual
Do your muscles hurt after exercise?	Less than usual	No more than usual	Worse than usual	Much worse than usual

If you are tired at the moment, please indicate approximately how long this has lasted.

Less than 1 week	Less than 3 months	Between 3 & 6 months	6 months or more

Overall, what percentage of the time do you feel tired?

25% of the time	50% of the time	75% of the time	All the time

Why do you think you are feeling tired?

Barthel Index.

Bowels

0 = incontinent (or need to be given enemata)
1 = occasional accident (once a week)
2 = continent

Bladder

0 = incontinent, or catheterised and unable to manage alone
1 = occasional accident (maximum once per 24 hours)
2 = continent

Grooming

0 = Need help with personal care
1 = Independent face/hair/teeth/shaving (implements provided)

Toilet use.

0 = Dependent
1 = Needs some help, but can do something alone
2 = Independent (on and off, dressing, wiping)

Feeding

0 = Unable
1 = Needs help cutting, spreading butter etc.
2 = Independent

Transfer (bed to chair and back)

0 = Unable, no sitting balance
1 = Major help (one or two people, physical), can sit
2 = Minor help (verbal or physical)
3 = Independent

Mobility

0 = Immobile
1 = Wheelchair independent, including corners
2 = Walks with help of one person (verbal or physical)
3 = Independent (but may use any aid; for example stick)

Dressing

0 = Dependent
1 = Needs help but can do about half unaided
2 = Independent (including buttons, zips, laces, etc.)

Stairs

0 = Unable
1 = Needs help (verbal, physical, carrying aid)
2 = Independent

Bathing

0 = Dependent
1 = Independent (or in a shower)

Total (0-20) ☐

Social Support Questionnaire (Six Item Short Form. SSQ6)

The following questions ask about people in your environment who provide you with help or support. Each question has two parts.

For the first part, list all the people you know, excluding yourself, whom you can count on for help or support in the manner described. Give the person's initials and their relationship to you (see example).

Do not list more than one person next to each of the letters beneath the question.

For the second part, circle how satisfied you are with the overall support you have. If you have no support for a question, tick the words "No one", but still rate your level of satisfaction.

Do not list more than nine persons per question

Please answer all questions as best you can. All your responses will be kept confidential.

EXAMPLE

Who do you know whom you can trust with information that could get you into trouble?

No one ☐

1) T.N. (brother)	4) T.N (father)	7)
2) L.M. (friend)	5) L.N. (employer)	8)
3) R.S. (friend)	6)	9)

How satisfied?

6- very satisfied	5- fairly satisfied	4- a little satisfied	3- a little dissatisfied	2- fairly dissatisfied	1- very dissatisfied

---

1. Whom can you really count on to distract you from your worries when you feel under stress?

No one ☐ 1) 2) 3) 4) 5) 6) 7) 8) 9)

How satisfied?

6- very satisfied | 5- fairly satisfied | 4- a little satisfied | 3- a little dissatisfied | 2- fairly dissatisfied | 1- very dissatisfied

---

2. Whom can you really count on to help you feel more relaxed when you are under pressure or tense?

No one ☐ 1) 2) 3) 4) 5) 6) 7) 8) 9)

How satisfied?

6- very satisfied | 5- fairly satisfied | 4- a little satisfied | 3- a little dissatisfied | 2- fairly dissatisfied | 1- very dissatisfied

---

3. Who accepts you totally, including both your worst and your best points?

No one ☐ 1) 2) 3) 4) 5) 6) 7) 8) 9)

How satisfied?

6- very satisfied | 5- fairly satisfied | 4- a little satisfied | 3- a little dissatisfied | 2- fairly dissatisfied | 1- very dissatisfied

4. Whom can you really count on to care about you, regardless of what is happening to you?

No one ☐ 1) 2) 3) 4) 5) 6) 7) 8) 9)

How satisfied?

6- very satisfied	5- fairly satisfied	4- a little satisfied	3- a little dissatisfied	2- fairly dissatisfied	1- very dissatisfied

---

5. Whom can you really count on to help you feel better when you are feeling generally down-in-the-dumps?

No one ☐ 1) 2) 3) 4) 5) 6) 7) 8) 9)

How satisfied?

6- very satisfied	5- fairly satisfied	4- a little satisfied	3- a little dissatisfied	2- fairly dissatisfied	1- very dissatisfied

---

6. Whom can you count on to console you when you are very upset?

No one ☐ 1) 2) 3) 4) 5) 6) 7) 8) 9)

How satisfied?

6- very satisfied	5- fairly satisfied	4- a little satisfied	3- a little dissatisfied	2- fairly dissatisfied	1- very dissatisfied

HAD SCALE

Reproduced for display only

Hospital Anxiety and Depression Scale (HADS)

NFER-NELSON
INFORMING YOUR DECISIONS

FOLD HERE

FOLD HERE

Name: _____ Date: _____

Clinicians are aware that emotions play an important part in most illnesses. If your clinician knows about these feelings he or she will be able to help you more.

This questionnaire is designed to help your clinician to know how you feel. Read each item below and **underline the reply** which comes closest to how you have been feeling in the past week. Ignore the numbers printed at the edge of the questionnaire.

Don't take too long over your replies, your immediate reaction to each item will probably be more accurate than a long, thought-out response.

A D

I feel tense or 'wound up'
- [3] Most of the time
- [2] A lot of the time
- [1] From time to time, occasionally
- [0] Not at all

I still enjoy the things I used to enjoy
- [0] Definitely as much
- [1] Not quite so much
- [2] Only a little
- [3] Hardly at all

I get a sort of frightened feeling as if something awful is about to happen
- [3] Very definitely and quite badly
- [2] Yes, but not too badly
- [1] A little, but it doesn't worry me
- [0] Not at all

I can laugh and see the funny side of things
- [0] As much as I always could
- [1] Not quite so much now
- [2] Definitely not so much now
- [3] Not at all

Worrying thoughts go through my mind
- [3] A great deal of the time
- [2] A lot of the time
- [1] Not too often
- [0] Very little

I feel cheerful
- [3] Never
- [2] Not often
- [1] Sometimes
- [0] Most of the time

I can sit at ease and feel relaxed
- [0] Definitely
- [1] Usually
- [2] Not often
- [3] Not at all

A

I feel as if I am slowed down
- Nearly all the time
- Very often
- Sometimes
- Not at all

I get a sort of frightened feeling like 'butterflies' in the stomach
- Not at all
- Occasionally
- Quite often
- Very often

I have lost interest in my appearance
- Definitely
- I don't take as much care as I should
- I may not take quite as much care
- I take just as much care as ever

I feel restless as if I have to be on the move
- Very much indeed
- Quite a lot
- Not very much
- Not at all

I look forward with enjoyment to things
- As much as I ever did
- Rather less than I used to
- Definitely less than I used to
- Hardly at all

I get sudden feelings of panic
- Very often indeed
- Quite often
- Not very often
- Not at all

I can enjoy a good book or radio or television programme
- Often
- Sometimes
- Not often
- Very seldom

Now check that you have answered all the questions

TOTAL

The Arthritis Body Experience Scale (ABES) (Williams & Barlow, 1998)
Adapted for Neuromuscular Disease

These statements relate to the **IMPACT of your MUSCLE DISEASE** on your **BODY**. Please **circle** the appropriate **number** to indicate your **agreement** or **disagreement** with each statement on a scale of **1 (strongly disagree)** to **10 (strongly agree).**

1. I am happy with my body

strongly disagree strongly agree

1 2 3 4 5 6 7 8 9 10

2. I am self-conscious about my body

strongly disagree strongly agree

1 2 3 4 5 6 7 8 9 10

3. I am self-conscious about the parts of my body affected by muscle disease that are visible to others

strongly disagree strongly agree

1 2 3 4 5 6 7 8 9 10

4. I wear particular clothing to hide certain parts of my body affected by my muscle disease

strongly disagree strongly agree

1 2 3 4 5 6 7 8 9 10

5. I am happy with my posture

strongly disagree strongly agree

1 2 3 4 5 6 7 8 9 10

6. I am embarrassed about the parts of my body affected by

my muscle disease

strongly disagree strongly agree

1 2 3 4 5 6 7 8 9 10

7. I am happy with the way I walk

strongly disagree strongly agree

1 2 3 4 5 6 7 8 9 10

8. My body is physically attractive

strongly disagree strongly agree

1 2 3 4 5 6 7 8 9 10

9. I am concerned with the physical fitness of my body

strongly disagree strongly agree

1 2 3 4 5 6 7 8 9 10